UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2024

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934 FOR THE TRANSITION PERIOD FROM TO**

Commission File Number : 001-14895

Sarepta Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**93-0797222**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification Number)**
215 First Street **Suite 415** **Cambridge, MA**	**02142**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 274-4000

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, $0.0001 par value	**SRPT**	**The NASDAQ Stock Market LLC (The NASDAQ Global Select Market)**

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the Registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. YES ☒ NO ☐

Indicate by check mark if the Registrant is not required to file reports pursuant to Section 13 or 15(d) of the Act. YES ☐ NO ☒

Indicate by check mark whether the Registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the Registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. YES ☒ NO ☐

Indicate by check mark whether the Registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the Registrant was required to submit such files). YES ☒ NO ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☒	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐
Emerging growth company	☐		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☒

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

Indicate by check mark whether the Registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). YES ☐ NO ☒

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the Registrant, based on the closing price of the shares of common stock on The Nasdaq Global Select Market on June 30, 2024, was approximately $15,054,650,800.

The number of shares of Registrant's Common Stock outstanding as of February 24, 2025 was 97,032,073.

DOCUMENTS INCORPORATED BY REFERENCE

The registrant has incorporated by reference into Part II and Part III of this Annual Report on Form 10-K portions of its definitive Proxy Statement for the 2025 Annual Meeting of Stockholders to be filed no later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K.

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Forward-Looking Information

This Annual Report on Form 10-K, including the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section in Item 7, and other materials accompanying this Annual Report on Form 10-K contain forward-looking statements or incorporate by reference forward-looking statements. Statements that are not purely historical are forward-looking statements. Forward-looking statements are often identified by words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may," "estimate," "could," "continue," "ongoing," "predict," "potential," "likely," "seek" and other similar expressions, as well as variations or negatives of these words. These statements address expectations, projections of future results of operations or financial condition, or other "forward-looking" information. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to:

- *our belief that our proprietary technology platforms and collaborations can be used to develop potential therapeutic candidates to treat a broad range of diseases;*
- *our expectation that our partnerships with manufacturers will support our clinical and commercial manufacturing capacity for our Duchenne muscular dystrophy ("Duchenne") gene therapy program and Limb-girdle muscular dystrophy ("LGMD") programs, while also acting as a manufacturing platform for potential future gene therapy programs, and our belief that our current network of manufacturing partners is able to fulfill the requirements of our commercial plan;*
- *our expectation that our partnership with Catalent Maryland, Inc. ("Catalent") will support our clinical and commercial manufacturing demand for our Duchenne gene therapy program and LGMD programs, while also acting as a manufacturing platform for potential future gene therapy programs;*
- *our expectation that Aldevron LLC ("Aldevron") will provide Good Manufacturing Processes ("GMP")-grade plasmid for our Duchenne gene therapy program and LGMD programs, as well as plasmid source material for future gene therapy programs;*
- *estimated timelines and milestones for 2025 and beyond, including submitting a SRP-9003 BLA in the second half of 2025;*
- *our plan to expand our pipeline through internal research and development and through strategic transactions;*
- *the timely completion and satisfactory outcome of our post-marketing requirements and commitments, including verification of a clinical benefit for our products;*
- *our engagement with regulatory authorities outside of the U.S.;*
- *the possible impact of regulations and regulatory decisions by the Food and Drug Administration (the "FDA") and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations;*
- *the possible impact of legislation and any competing products on the commercial success of our products and our product candidates and our ability to compete against such products;*
- *our ability to enter into research, development or commercialization alliances with universities, hospitals, independent research centers, non-profit organizations, pharmaceutical and biotechnology companies and other entities for specific molecular targets or selected disease indications and our ability to selectively pursue opportunities to access certain intellectual property rights that complement our internal portfolio through license agreements or other arrangements;*
- *our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future;*
- *the potential benefits of our technologies and programs, including those with strategic partners;*
- *our plans and ability to file and progress to issue additional patent applications to enhance and protect our new and existing technologies and programs;*
- *our estimates regarding how long our currently available cash and cash equivalents will be sufficient to finance our operations and business plans and statements about our future capital needs;*
- *our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;*
- *our expectation regarding the impact of environmental laws and regulations on our business; and*

- *our beliefs and expectations regarding milestone, royalty or other payments that could be due to third parties under existing agreements.*

We undertake no obligation to update any of the forward-looking statements contained in this Annual Report on Form 10-K after the date of this report, except as required by law or the rules and regulations of the U.S. Securities and Exchange Commission (the "SEC"). We caution readers not to place undue reliance on forward-looking statements. Our actual results could differ materially from those discussed in this Annual Report on Form 10-K. The forward-looking statements contained in this Annual Report on Form 10-K, and other written and oral forward-looking statements made by us from time to time, are subject to certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements, including the risks, uncertainties and assumptions identified under the heading "Risk Factors" in this Annual Report on Form 10-K.

Risk Factors Summary

Our business is subject to numerous risks and uncertainties, including those described in Item 1A "Risk Factors". These risks include, but are not limited to the following:

- We are highly dependent on the commercial success of our products in the U.S. We may not be able to meet expectations with respect to sales of our products or maintain profitability and positive cash-flow from operations.
- Even though EXONDYS 51, VYONDYS 53, AMONDYS 45 and ELEVIDYS (with respect to non-ambulatory patients) have received accelerated approval from the FDA, they face future post-approval development and regulatory requirements, which present additional challenges for us to successfully navigate.
- Failure to obtain or maintain regulatory exclusivity for our products could result in our inability to protect our products from competition and our business may be adversely impacted.
- We are subject to uncertainty relating to reimbursement policies which, if not favorable, could hinder or prevent the commercial success of our products and/or product candidates.
- Our products may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our potential profitability and future business prospects.
- Historical revenues from eteplirsen, golodirsen and casimersen through our early access program ("EAP") outside the U.S. may not continue and we may not be able to continue to distribute our products through our EAP.
- We face intense competition and rapid technological change, which may result in other companies discovering, developing or commercializing competitive products.
- We have entered into multiple collaborations and strategic transactions, including our collaboration with F. Hoffman-La Roche Ltd ("Roche") and our collaboration and license agreement with Arrowhead Pharmaceuticals, Inc. ("Arrowhead"), and may seek or engage in future collaborations, strategic alliances, acquisitions or licensing agreements that complement or expand our business. We may not be able to complete such transactions, and such transactions, if executed, may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.
- We may find it difficult to enroll patients in our clinical trials, which could delay or prevent clinical trials of our product candidates.
- Future sales of ELEVIDYS may decrease sales growth, or reduce sales, of EXONDYS 51, AMONDYS 45 and VYONDYS 53 (collectively, the "PMO Products"), which could negatively impact our operating results, including through potential inventory write-offs.
- Failures or delays in the commencement or completion of ongoing and planned clinical trials of our product candidates could negatively impact commercialization efforts; result in increased costs; and delay, prevent or limit our ability to gain regulatory approval of product candidates and to generate revenues and continue our business.
- Clinical development is lengthy and uncertain. Clinical trials of our product candidates may be delayed and certain programs may never advance in the clinic or may be more costly to conduct than we anticipate, any of which could have a material adverse impact on our business.
- Results from pre-clinical and early-stage clinical trials may not be indicative of efficacy in late-stage clinical trials, and pre-clinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, which could prevent or significantly delay their regulatory approval.
- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent regulatory approval of product candidates, limit the commercial potential or result in significant negative consequences following any

potential marketing approval.

- If there are significant delays in obtaining, or if we are unable to obtain or maintain required regulatory approvals, we will not be able to commercialize our product candidates in a timely manner or at all.

- We are investing significant resources in the development of novel gene therapy product candidates. Only a few gene therapy products have been approved in the U.S. and the European Union ("EU"). If we are unable to show the safety and efficacy of these product candidates, experience delays in doing so or are unable to successfully commercialize these drugs, our business would be materially harmed.

- Because we are developing product candidates for the treatment of certain diseases in which there is little clinical experience and we are using new endpoints or methodologies, there is increased risk that the FDA, the European Medicines Agency (the "EMA") or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent full or accelerated regulatory approval.

- We may not be able to advance all of our programs, and we may use our financial and human resources to pursue particular programs and fail to capitalize on programs that may be more profitable or for which there is a greater likelihood of success.

- If we are unable to maintain our agreements with third parties to distribute our products to patients, our results of operations and business could be adversely affected.

- We rely on third parties to conduct some aspects of our early stage research and pre-clinical and clinical development. The inadequate performance by or loss of any of these third parties could affect the development and commercialization of our product candidate development. The third parties we use in the manufacturing process for our products and product candidates may fail to comply with current good manufacturing practices ("cGMP") regulations.

- We currently rely on third parties to manufacture our products and to produce our product candidates. Our dependence on these parties, including failure on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet commercial, EAP, clinical and pre-clinical product demand may impair the availability of product to successfully support various programs, including research and development and the potential commercialization of additional product candidates in our pipeline.

- Products intended for use in gene therapies are novel, complex and difficult to manufacture. We could experience production problems or inaccurately forecast demand for any of our product candidates, which could result in delays in our development or commercialization, limit supply or otherwise harm our business.

- Our success, competitive position and future revenue depend in part on our ability and the abilities of our licensors and other collaborators to obtain, maintain and defend the patent protection for our products, product candidates, and platform technologies, to preserve our trade secrets, and to prevent third parties from infringing on our proprietary rights.

- Our stock price is volatile and may fluctuate due to factors beyond our control.

- Our existing and any future indebtedness could adversely affect our ability to operate our business.

- Our revenues and operating results could fluctuate significantly, which may adversely affect our stock price and our ability to maintain profitability.

PART I

Item 1. Business.

Overview

We are a commercial-stage biopharmaceutical company focused on helping patients through the discovery and development of unique RNA-targeted therapeutics, gene therapy and other genetic therapeutic modalities for the treatment of rare diseases. Applying our proprietary, highly-differentiated and innovative technologies, and through collaborations with our strategic partners, we have developed multiple approved products for the treatment of Duchenne and are developing potential therapeutic candidates for a broad range of diseases and disorders, including Duchenne, LGMDs, other neuromuscular disorders, such as facioscapulohumeral muscular dystrophy ("FSHD") and myotonic dystrophy type 1 ("DM1"), and central nervous system ("CNS") related disorders.

To date, we have developed and commercialized the following four approved products for the treatment of Duchenne: EXONDYS 51 (eteplirsen) Injection ("EXONDYS 51"), VYONDYS 53 (golodirsen) Injection ("VYONDYS 53"), AMONDYS 45 (casimersen) Injection ("AMONDYS 45"), and ELEVIDYS. Each of these approved products, and the indications for which they have been approved for, is described under the heading "Our Commercial Products" in this Item 1.

Objectives and Business Strategy

We believe that our proprietary technology platforms and collaborations can be used to develop novel pharmaceutical products to treat a broad range of diseases and address key currently-unmet medical needs. We intend to leverage our technology platforms, organizational capabilities, collaborations and resources to lead the field of precision genetic medicines, including the treatment of rare, neuromuscular and other diseases, with a diversified portfolio of product candidates. In pursuit of this objective, we intend to focus on the following activities:

- continuing to build our gene therapy engine, including developing gene therapy product candidates, operationalizing our manufacturing strategy and furthering our commercial capabilities in preparation for potential regulatory approvals;
- advancing our RNA technologies, launching potential approved products and supporting commercialization of approved products;
- investing in next-generation precision medicine through internal research, strategic partnerships, collaborations and other potential opportunities; and
- continuing to nurture our culture, which is based on strong patient focus, bias to action, a self-starter mentality, smart and appropriate risk-taking and high ethics.

Technology and Platforms

- Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein. The original phosphorodiamidate morpholino oligomer ("PMO") structure and variations of this structure that are so-called PMO-based (collectively "PMO-based") are central to our proprietary chemistry platform. PMO technologies can be used to selectively up-regulate or down-regulate the production of a target protein through pre-mRNA splice alteration. PMO-based compounds have the potential to be designed to create more, less, or none of certain proteins, or produce analogues of endogenous proteins. This technology can be used to correct disease-causing genetic errors by inducing the targeted expression of novel proteins.
- As part of our multifaceted approach to Duchenne, we are also developing gene therapy technologies to treat Duchenne. Our gene therapy aims to express a smaller but still functional version of dystrophin. We use a unique adeno-associated virus ("AAV") vector called AAVrh.74 to transport the transgene – the genetic material that will make the protein of interest – to the target cells. A unique, engineered dystrophin is used because naturally-occurring dystrophin is too large to fit in an AAV. We are also developing gene therapy programs for various forms of LGMDs. Our most advanced LGMD product candidate, SRP-9003, is designed to transfer a gene that codes for and restores beta-sarcoglycan protein with the goal of restoring the dystrophin associated protein complex. SRP-9003 utilizes the AAVrh.74 vector, the same vector used in ELEVIDYS.

Our pipeline includes programs at various stages of discovery, pre-clinical and clinical development. Through our collaborations with our strategic partners, we are expanding into adjacent therapeutic areas. Our pipeline reflects our aspiration to apply our multifaceted approach and expertise in precision genetic medicine to make a profound difference in the lives of patients suffering from rare diseases.

Core Therapeutic Areas

Duchenne: Duchenne is a rare X-linked recessive genetic disorder affecting children (primarily males) that is characterized by progressive muscle deterioration and weakness. It is the most common type of muscular dystrophy. Duchenne is caused by an absence of dystrophin, a protein that protects muscle cells. The absence of dystrophin in muscle cells leads to significant cell damage and ultimately causes muscle cell death and fibrotic replacement. In the absence of dystrophin protein, affected individuals generally experience the following symptoms, although disease severity and life expectancy vary:

- muscle damage characterized by inflammation, fibrosis and loss of myofibers beginning at an early age;
- muscle weakness and progressive loss of muscle function beginning in the first few years of life;
- decline of ambulation and respiratory function after the age of seven;
- total loss of ambulation in the pre-teenage or early teenage years;
- progressive loss of upper extremity function during mid- to late-teens; and
- respiratory and/or cardiac failure, resulting in death before the age of 30.

LGMDs are autosomal recessive, monogenic, rare neuromuscular diseases caused by missense and deletion mutations. These diseases affect males and females equally. Some types of LGMDs affect skeletal muscle and cardiac muscle. More severe forms of LGMDs mimic Duchenne. LGMDs as a class affect an estimated range of approximately 1 in every 14,500 to 1 in every 123,000 individuals. Currently, there are no approved treatment options for LGMDs.

Charcot-Marie-Tooth ("CMT") Disease is a group of hereditary, degenerative nerve diseases that are caused by mutations in genes that produce proteins involved in the structure and function of either the peripheral nerve axon or the myelin sheath. CMT can cause degeneration of motor skills, resulting in muscle weakness, and limiting patients' ability to walk or use their hands, and in some cases, can cause degeneration of sensory nerves, resulting in a reduced ability to feel heat, cold, and pain. CMT affects approximately 1 in every 2,500 individuals, while CMT type 1A, which is most often caused by an extra copy of the PMP22 gene, affects approximately 50,000 patients in the U.S. Most patients are diagnosed at infancy, while other patients develop symptoms at adolescence. Currently, there are no available treatment options.

Other Neuromuscular Disorders: We, and through our strategic collaborations with partners, are exploring a wide range of neuromuscular disorders, including facioscapulohumeral muscular dystrophy and myotonic dystrophy.

Our Commercial Products

EXONDYS 51. We launched our first commercial product, EXONDYS 51, in 2016. EXONDYS 51 is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 51 skipping. EXONDYS 51 uses our PMO chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. PMO-based compounds are synthetic compounds that bind to complementary sequences of RNA by standard Watson-Crick nucleobase pairing. The two key structural differences between PMO-based compounds and naturally occurring RNA are that the PMO nucleobases are bound to synthetic morpholino rings instead of ribose rings, and the morpholino rings are linked by phosphorodiamidate groups instead of phosphodiester groups. Replacement of the negatively charged phosphodiester in RNA with the uncharged phosphorodiamidate group in PMO eliminates linkage ionization at physiological pH. Due to these modifications, PMO-based compounds are resistant to degradation by plasma and intracellular enzymes. Unlike the RNA-targeted technologies such as siRNAs and DNA gapmers, PMO-based compounds operate by steric blockade rather than by cellular enzymatic degradation to achieve their biological effects. Thus, PMOs use a fundamentally different mechanism from other RNA-targeted technologies.

EXONDYS 51 targets the most frequent series of mutations that cause Duchenne. Approximately 13% of Duchenne patients are amenable to exon 51 skipping.

VYONDYS 53. We launched VYONDYS 53 in 2019. VYONDYS 53 is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 53 skipping. VYONDYS 53 uses our PMO chemistry and exon-skipping technology to skip exon 53 of the dystrophin gene. Approximately 8% of Duchenne patients are amenable to exon 53 skipping.

AMONDYS 45. We launched AMONDYS 45 in 2021. AMONDYS 45 is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 45 skipping. AMONDYS 45 uses our PMO chemistry and exon-skipping technology to skip exon 45 of the dystrophin gene. Approximately 8% of Duchenne patients are amenable to exon 45 skipping.

We are conducting various clinical trials for EXONDYS 51, VYONDYS 53 and AMONDYS 45, including studies that are required to comply with our post-marketing FDA requirements and commitments to verify and describe the clinical benefit of the three products.

ELEVIDYS. We launched ELEVIDYS in the second quarter of 2023. ELEVIDYS, an AAV-based gene therapy, was approved by the FDA in June 2024 for the treatment of ambulatory patients at least four years old with Duchenne with a confirmed mutation in the Duchenne gene. ELEVIDYS is also approved for non-ambulatory patients under the accelerated approval pathway. ELEVIDYS was previously granted accelerated approval by the FDA in June 2023 for the treatment of ambulatory patients aged four through five years with Duchenne with a confirmed mutation in the Duchenne gene. ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the Duchenne gene.

We are conducting various clinical trials for ELEVIDYS, including studies that are required to comply with our post-marketing FDA requirements and commitments to verify and describe clinical benefit.

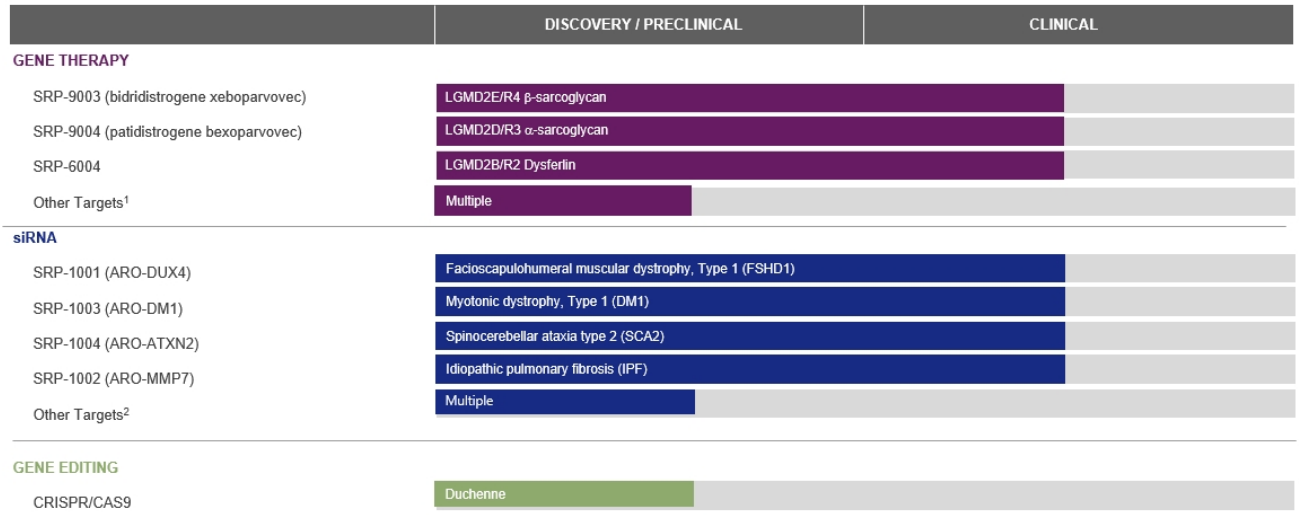
For the years ended December 31, 2024, 2023 and 2022, we recorded net revenues of $1,788.0 million, $1,144.9 million and $843.8 million, respectively, related to the sale of our products.

Our Pipeline – Key Programs

SRP-9003 (LGMD, gene therapy program). The most advanced of our LGMD product candidates, SRP-9003, aims to treat LGMD2E, also known as beta-sarcoglycanopathy, a severe and debilitating form of LGMD characterized by progressive muscle fiber loss, inflammation and muscle fiber replacement with fat and fibrotic tissue. SRP-9003 is designed to transfect a gene that codes for and restores beta-sarcoglycan protein with the goal of restoring the dystrophin associated protein complex. SRP-9003 has generated positive pre-clinical safety and efficacy data utilizing the AAVrh.74 vector, the same vector used in our SRP-9001 gene therapy program.

A Phase 1/2a trial of SRP-9003 commenced in the fourth quarter of 2018. In June 2020, we announced safety and expression results from three clinical trial participants in the high-dose cohort measured at 60 days, and one-year functional data from three clinical trial participants in the low-dose cohort. In March 2022, we announced 36-month functional data from three clinical trial participants in the low-dose cohort and 24-month functional data from two clinical trial participants in the high-dose cohort. In December 2024, we announced that we had completed enrollment and dosing in EMERGENE (Study SRP-9003-301), a Phase 3 clinical trial of SRP-9003 (bidridistrogene xeboparvovec).

The chart below summarizes the status of our programs, including those with our strategic partners:



1. Other gene therapies comprise muscular dystrophy, neuro and cardiac indications including SRP-6006 (LGMD2B/R2 dysferlin), SRP-9005 (LGMD2C/R5 γ-sarcoglycan), SRP-9006 (LGMD2L/R12 Anoctamin 5), SRP-9010 (LGMD2A/R1 calpain-3-related) and Charcot-Marie-Tooth
2. Other siRNA indications include Huntington's disease, SCA1 and SCA3

Manufacturing, Supply and Distribution

We have developed proprietary state-of-the-art Chemistry, Manufacturing and Controls ("CMC") capabilities that allow manufacturing and testing of our products and product candidates to support both clinical development and commercialization. We continue to refine and optimize our manufacturing processes and test methods. We have entered into certain manufacturing and supply arrangements with third-party suppliers which will in part utilize these capabilities to support production of certain of our product candidates and their components. We have also opened facilities over the past several years that significantly enhanced our internal research and development capabilities. However, we currently do not have internal GMP manufacturing capabilities to produce our products and product candidates for commercial and/or clinical use. For our current and future manufacturing needs, we have entered into supply agreements with specialized contract manufacturing organizations (each a "CMO") to produce custom raw materials, the active pharmaceutical ingredients ("APIs"), drug product and finished goods for our products and product candidates for both commercial and clinical use. All of our CMO partners have extensive technical expertise, GMP experience and experience manufacturing our specific technology.

For our commercial Duchenne PMO program, we have worked with our existing CMOs to increase production capacity from mid-scale to large-scale. While there is a limited number of companies that can produce raw materials and APIs in the quantities and with the quality and purity that we require for our commercial products, based on our diligence to date, we believe our current network of CMOs is able to fulfill these requirements, and is capable of expanding capacity as needed. Additionally, we have evaluated, and will continue to evaluate further relationships with additional suppliers to increase overall capacity as well as further reduce risks associated with relying on a limited number of suppliers for manufacturing.

Our gene therapy manufacturing capabilities have been greatly enhanced through partnerships with Aldevron and Catalent. We have adopted a hybrid development and manufacturing strategy in which we have built internal expertise relative to all aspects of AAV-based manufacturing, including gene therapy and gene editing, while closely partnering with experienced manufacturing partners to expedite development and commercialization of our gene therapy programs. We have secured manufacturing capacity at Catalent to support our clinical and commercial manufacturing demand for ELEVIDYS and our LGMD programs, while also acting as a manufacturing platform for potential future gene therapy programs. The collaboration integrates process development, clinical and commercial production and testing. Aldevron provides GMP-grade plasmid for ELEVIDYS and is expected to provide plasmid source material for our LGMD programs and future gene therapy programs.

Manufacturers and suppliers of our commercial products and product candidates are subject to the current GMP ("cGMP") requirements and other rules and regulations prescribed by the FDA and other foreign regulatory authorities. We depend on our third-party partners for continued compliance with cGMP requirements and applicable foreign standards.

Our PMO commercial products are distributed in the U.S. through a limited network of home infusion specialty pharmacy providers that deliver the medication to patients and a specialty distributor that distributes our products to hospitals and hospital outpatient clinics. With respect to the pre-commercial distribution of our products to patients outside of the U.S., we have contracted with third party distributors and service providers to distribute our products in certain countries through our EAPs. We plan to continue building out our network for commercial distribution in jurisdictions in which our products are approved.

The U.S. distribution model for ELEVIDYS employs multiple distribution partners that include third-party logistics providers, as well as a limited network of specialty pharmacy providers that provide the medication to hospitals for infusion.

Material Agreements

We believe that our RNA-targeted and gene therapy technologies could be broadly applicable for the potential development of pharmaceutical products in many therapeutic areas. To enhance and further exploit our core technologies, we have and may continue to enter into research, development or commercialization alliances with universities, hospitals, independent research centers, non-profit organizations, pharmaceutical and biotechnology companies and other entities for new technologies, including for specific molecular targets or selected disease indications. We may also selectively pursue opportunities to access certain intellectual property rights that complement our internal portfolio through license agreements or other arrangements.

The following descriptions of the terms of our material agreements is not complete and is qualified in their entirety by reference to the text of the agreements, copies of which are filed as exhibits to this Annual Report.

F. Hoffman-La Roche Ltd

License, Collaboration, and Option Agreement

On December 21, 2019, we entered into a license, collaboration, and option agreement (the "Roche Agreement") with F. Hoffman-La Roche Ltd ("Roche") pursuant to which we granted Roche an exclusive license under certain of our intellectual property rights to develop, manufacture, and commercialize ELEVIDYS (SRP-9001) in all countries outside of the U.S. We retained all rights

to ELEVIDYS in the U.S. The transaction closed on February 4, 2020. We have subsequently entered into Amendments 1 through 14 to the Roche Agreement on: October 23, 2020, October 28, 2020, February 4, 2021, June 23, 2021, August 31, 2021, November 30, 2021, January 5, 2022, January 28, 2022, March 23, 2022, May 31, 2022, June 23, 2022, July 28, 2022, August 31, 2022 and October 31, 2022, respectively.

Also, under the terms of the Roche Agreement, Roche granted us a license to use certain of its intellectual property rights to perform development activities worldwide under a joint global development plan, commercialize ELEVIDYS in the U.S., and perform certain manufacturing and medical affairs activities worldwide. Such license is non-exclusive under Roche's background intellectual property rights, exclusive in the U.S. under intellectual property rights developed by Roche under the Roche Agreement, and non-exclusive outside the U.S. under intellectual property rights developed by Roche under the Roche Agreement.

We intend to manufacture and supply ELEVIDYS in the relevant markets in which we have approval, or in the future receive approval.

Roche Options and Negotiation Rights

Pursuant to the Roche Agreement, we granted Roche an exclusive option to obtain an exclusive license to develop, manufacture and commercialize the following products outside of the U.S.: (i) certain exon-skipping products that target the dystrophin gene to induce exon skipping, including eteplirsen, golodirsen, casimersen (and previously our SRP-5051 program); (ii) certain gene therapy products other than ELEVIDYS that encode and directly express dystrophin or a derivative thereof, which right has since expired; and (iii) certain gene-editing products that modify, repair, or activate an endogenous dysfunctional dystrophin gene, which right has since expired (collectively, the "Option Products.") Upon option exercise, the Option Product that is the subject of the option exercise will be included under the Roche Agreement as a product licensed to Roche subject to similar obligations, including with respect to development, manufacturing, commercialization, and cost-sharing as those that apply to ELEVIDYS.

Pursuant to the Roche Agreement, Roche has a right of first negotiation if we seek to grant a third-party license to commercialize ELEVIDYS in the U.S. Roche had a similar right of first negotiation with respect to our LGMDs products, but such right has expired.

Exclusivity

Other than under the Roche Agreement, Roche may not perform any clinical trials for, or commercialize, any gene therapy product, gene-editing product, or antisense oligonucleotide for Duchenne for a period of five years following the effective date of the Roche Agreement, which obligation has since expired with respect to gene therapy products and gene-editing products. The exclusivity period for antisense oligonucleotide products will be extended for a period of five years from the time of option exercise if Roche exercises its option with respect to exon skipping products.

Development

The parties will use commercially reasonable efforts to conduct development activities with respect to ELEVIDYS under the Roche Agreement pursuant to agreed-upon development plans. Subject to certain exceptions, we will perform all development activities directed to obtaining and maintaining, as applicable, regulatory approvals for ELEVIDYS in the U.S. and the EU, as set forth in a joint global development plan. Subject to certain exceptions, the parties will share the costs of the development activities under such joint global development plan. Roche will have sole responsibility to perform all development activities set forth in a territory-specific development plan for ELEVIDYS, including additional activities not set forth in the joint global development plan that are specifically directed to obtaining and maintaining regulatory approvals for ELEVIDYS outside of the U.S. Roche will be solely responsible for costs arising from the territory-specific development plan for ELEVIDYS.

Governance

Governing committees have been established to facilitate collaboration between the parties with respect to development, manufacturing, medical affairs, intellectual property protection, and commercialization of ELEVIDYS and any other licensed products.

Financial Terms

In consideration for the rights that we granted and for prepaid funding for development activities, in February 2020, Roche and Roche Finance Ltd, an affiliate of Roche ("Roche Finance"), together paid us an up-front payment of approximately $1.2 billion. Of the $1.2 billion cash received from Roche, (i) $312.1 million, net of issuance costs, was allocated to the approximately 2.5 million shares of our common stock issued to Roche based on the closing price when the shares were issued, (ii) $485.0 million was allocated

to the option to purchase the Option Products, and (iii) $348.7 million was allocated to a single, combined performance obligation comprised of: (i) the license of IP relating to ELEVIDYS transferred to Roche, (ii) the related research and development services provided under the joint global development plan, (iii) the services provided to manufacture clinical supplies of ELEVIDYS, and (iv) our participation in a joint steering committee with Roche, because we determined that the license of IP and related activities were not capable of being distinct from one another. Additionally, we are eligible to receive up to $1.7 billion in development, regulatory and sales milestone payments with respect to ELEVIDYS.

In addition, the Roche Agreement provides that Roche will pay us royalties on net sales of ELEVIDYS, at a tiered royalty rate based on the average cost to manufacture ELEVIDYS.

In the event that Roche chooses to exercise its option with respect to one or more Option Products, we will be paid an option exercise fee upon each such exercise and the Option Products that are the subject of the option exercise will be subject to separate milestone payments and royalties on sales of such Option Product.

Term; Termination

Unless earlier terminated as described below, the Roche Agreement will continue with respect to ELEVIDYS or any Option Product for which Roche has exercised its option, on a product-by-product and country-by-country basis, until the end of the royalty term for such product in such country. The royalty term expires on the later of (a) twelve years after first commercial sale in such country, (b) loss of regulatory exclusivity in such country and (c) expiration of all valid claims of specific licensed patents in such country.

Either party may terminate the Roche Agreement for the other party's material breach if such breach is not cured within a specified cure period.

If Roche breaches its development or commercialization diligence obligations with respect to a licensed product or fails to develop or commercialize a particular licensed product in a particular region for a specified period of time, then we may terminate the Roche Agreement with respect to such licensed products in such regions.

Roche may terminate the Roche Agreement if we fail to supply ELEVIDYS to Roche in accordance with the terms of the Roche Agreement and the supply agreements to be entered into between the parties. Roche may also terminate the Roche Agreement for convenience with extended advance notice, in its entirety or on a licensed product-by-licensed product and region-by-region basis.

Myonexus Therapeutics Inc.

In April 2019, we acquired Myonexus Therapeutics Inc. ("Myonexus"), a privately-held Delaware corporation, for $173.8 million pursuant to an exclusive warrant to purchase Myonexus that we purchased in May 2018 for an up-front payment of $60.0 million. As part of the consideration for the transaction, we are required to make contingent payments to the former shareholders of Myonexus upon achievement of a threshold amount of net sales of Myonexus products and the receipt and subsequent sale of a Priority Review Voucher ("PRV") with respect to a Myonexus product.

BioMarin Pharmaceutical Inc. ("BioMarin")

License Agreement

On July 17, 2017, we executed a license agreement (as amended on April 14, 2019 and November 17, 2021, the "License Agreement") with BioMarin Leiden Holding BV, BioMarin Nederlands BV and BioMarin Technologies BV (collectively, the "BioMarin Parties"), pursuant to which BioMarin Parties granted us a royalty-bearing, worldwide license under patent rights ("Licensed Patents") and know-how ("Licensed Know-How") controlled by the BioMarin Parties with respect to BioMarin's Parties' Duchenne program, which are potentially necessary or useful for the treatment of Duchenne, to practice and exploit the Licensed Patents and Licensed Know-How in all fields of use and for all purposes, including to develop and commercialize antisense oligonucleotide products that target one or more exons of the dystrophin gene to induce exon skipping, including eteplirsen, golodirsen and casimersen (collectively, the "Products").

The license granted to us by the BioMarin Parties is co-exclusive with the BioMarin Parties, with respect to the Licensed Patents, and is non-exclusive with respect to Licensed Know-How. Pursuant to the amendment to the License Agreement dated November 17, 2021 (the "2021 Amendment"), the BioMarin Parties exercised their rights to convert the exclusive license under the Licensed Patents to the current co-exclusive license.

Under the terms of the License Agreement, we were required to pay the BioMarin Parties an up-front payment of $15.0 million. Pursuant to the 2021 Amendment, the BioMarin Parties are eligible to receive up to $20.0 million from us per dystrophin gene exon (other than exon 51) targeted by one or more Products in specified regulatory milestones, as well as an additional $10.0 million milestone, payable following the regulatory approval of eteplirsen by the EMA. The BioMarin Parties were also eligible to receive through June 30, 2022 royalties segmented by specified geographic markets, in some jurisdictions dependent on the existence of a patent, ranging from 4% to 8% of net sales on a product-by-product and country-by-country basis. Beginning July 1, 2022, pursuant to the 2021 Amendment, the BioMarin Parties were eligible to receive royalties of 4% in the U.S. and 5% outside the U.S. of net sales of Products covered by a Licensed Patent on a product-by-product and country-by-country basis.

Milestones and royalties were payable with respect to the Products through June 30, 2022. Beginning July 1, 2022, pursuant to the 2021 Amendment, milestones and royalties are payable only with respect to the Products covered by a Licensed Patent. Beginning July 1, 2022, pursuant to the 2021 Amendment, the royalty term applicable to the Products covered by a Licensed Patent expired upon March 31, 2024 in the U.S. and December 31, 2024 outside the U.S. The royalties for all Products covered by a Licensed Patent were subject to reductions, including for generic competition and, under specified conditions, for a specified portion of payments that we may become required to pay under third-party license agreements, subject to a maximum royalty reduction.

The License Agreement expired upon the expiration of the last-to-expire royalty term. We retain a royalty free, fully paid license to the Licensed Patents.

Settlement Agreement

On July 17, 2017, Sarepta and The University of Western Australia (“UWA”) on the one hand, and the BioMarin Parties and Academisch Ziekenhuis Leiden (“AZL”) on the other hand (collectively, the “Settlement Parties”), executed a Settlement Agreement (the "Settlement Agreement") pursuant to which all legal actions in the U.S. and certain legal actions in Europe (the “Actions”) would be stopped or withdrawn as between the Settlement Parties. Specifically, the terms of the Settlement Agreement required that existing efforts pursuing ongoing litigation and opposition proceedings would be stopped as between the Settlement Parties, and the Settlement Parties would cooperate to withdraw the Actions before the European Patent Office (except for actions involving third parties), the U.S. Patent and Trademark Office (“USPTO”), the U.S. Court of Appeals for the Federal Circuit and the High Court of Justice of England and Wales, except for the cross-appeal of the Interlocutory Decision of the Opposition Division dated April 15, 2013 of the European Patent Office of EP 1619249B1 in which Sarepta agreed to withdraw its appeal and the BioMarin Parties/AZL agreed to continue with its appeal with Sarepta having oversight of the continued appeal by the BioMarin Parties/AZL.

Additionally, under the terms of the Settlement Agreement, the Settlement Parties agreed to release each other and the customers, end-users, agents, suppliers, distributors, resellers, contractors, consultants, services and partners of Sarepta or the BioMarin Parties (as applicable) from claims and damages related to (i) the patent rights controlled by the releasing party that are involved in the Actions, (ii) with respect to Sarepta and UWA, its patent rights related to the patent rights involved in the Actions, and (iii) with respect to the BioMarin Parties and AZL, all of the Licensed Patents and Licensed Know-How.

Under the terms of the Settlement Agreement, we made an up-front payment of $20.0 million to the BioMarin Parties.

University of Western Australia

In April 2013, we entered into an agreement with UWA under which an existing exclusive license agreement between the two parties was amended and restated and, in June 2016, we entered into the first amendment to the license agreement (the “UWA License Agreement”). The UWA License Agreement grants us specific rights to compounds for the treatment of Duchenne by inducing exon skipping. EXONDYS 51, VYONDYS 53 and AMONDYS 45 fall under the scope of the license agreement. Under the UWA License Agreement, we are required to make payments of up to $6.0 million in the aggregate to UWA based on the successful achievement of certain development and regulatory milestones relating to EXONDYS 51, VYONDYS 53, AMONDYS 45 and up to three additional product candidates. As of December 31, 2024, $4.2 million of the $6.0 million development and regulatory milestone payments had been made. We are also obligated to make payments to UWA of up to $20.0 million upon the achievement of certain sales milestones. Additionally, we are required to pay a low-single-digit percentage royalty on net sales of products covered by issued patents licensed from UWA during the term of the UWA License Agreement.

Currently, the latest date on which an issued patent covered by the UWA License Agreement expires in November 2030 (excluding any patent term extension, supplemental protection certificate or pediatric extensions that may be available); however, patents granted from pending patent applications could result in a later expiration date.

Catalent Maryland, Inc.

Catalent Supply Agreement

On November 28, 2022, we entered into an amended and restated product manufacturing and supply agreement with Catalent. Under the Catalent Supply Agreement, Catalent has agreed, to manufacture and supply ELEVIDYS. Catalent is responsible for the operation of dedicated clean room suites for the manufacture of ELEVIDYS subject to Sarepta placing minimum annual orders. Catalent may not develop or manufacture products that compete with ELEVIDYS.

Supply Terms and Quality Assurance

The Catalent Supply Agreement contains customary supply terms, including requirements for forecasting, purchase orders, product specifications, batch testing and review procedures, price, payment terms, delivery mechanics and product insurance. In addition, it contains, a grant to Catalent of certain limited license rights of our intellectual property in connection with Catalent's performance of services under the Catalent Supply Agreement, certain indemnification rights in favor of both parties, and limitations of liability. We and Catalent have also entered into a quality agreement, pursuant to which Catalent will conduct certain quality assurance, testing, characterization, stability and other quality control procedures in connection with the manufacture and supply of our ELEVIDYS product under the Catalent Supply Agreement.

Financial Terms

Upon receipt of a purchase order from us, Catalent will manufacture ELEVIDYS in accordance with the terms of the Catalent Supply Agreement, the then-current quality agreement and any applicable laws in exchange for the batch price specified in the Catalent Supply Agreement, which may be increased annually for industry standard cost increases.

We are obligated to meet certain minimum annual thresholds with respect to orders of batches of ELEVIDYS to maintain dedicated manufacturing space, and, if we do not release the dedicated manufacturing space, we may be obligated to make certain payments to Catalent to the extent we do not meet such thresholds.

Term; Termination

Unless earlier terminated as described below, the Catalent Supply Agreement will continue with respect to the manufacture and supply of ELEVIDYS until December 31, 2028.

Either party may terminate the Catalent Supply Agreement for the other party's material breach, if such breach is not cured within a specified cure period, and in the event that the other party files a petition in bankruptcy, insolvency, or for reorganization or similar arrangement for the benefit of creditors, in the event the other party is served with an involuntary petition against it in any insolvency proceeding and such involuntary petition has not been stayed or dismissed within a specified timeframe, or in the event that the other party makes an assignment of substantially all of its assets for the benefit of creditors.

We may also terminate the Catalent Supply Agreement in the event of certain delivery or supply failures, involuntary market withdrawals, material safety risks, a change of control by Catalent without our prior written consent, or certain patent disputes, among other things.

There can be no assurance that we will be able to continue our present arrangement with Catalent. Our dependence upon our arrangement with Catalent for the supply and manufacture of ELEVIDYS could adversely affect our ability to manufacture and deliver ELEVIDYS on a timely and competitive basis. See "Risk Factors— Risks Related to Manufacturing."

Nationwide Children's Hospital

On October 8, 2018, we entered into an exclusive license agreement (as amended on May 19, 2019 and July 11, 2023, the "Nationwide License Agreement") with Nationwide Children's Hospital ("Nationwide") pursuant to which we acquired an exclusive license under certain intellectual property rights to develop, manufacture and commercialize ELEVIDYS in all countries. We entered into Amendment 1 and Amendment 2 to the Nationwide License Agreement on May 29, 2019 and July 11, 2023, respectively.

In consideration for the rights that Nationwide granted to us, we made an up-front payment and are obligated to make payments to Nationwide upon the achievement of certain development and sales milestones with respect to ELEVIDYS. In addition, we are required to pay a low-single-digit percentage royalty on net sales of ELEVIDYS, as well as a tiered percentage of remuneration

we receive in connection with any sublicenses we grant. Unless earlier terminated, the Nationwide License Agreement will expire upon the expiration of the last-to-expire royalty period. Either party may terminate the Nationwide License Agreement in the event of the other party's uncured material breach. Nationwide may also terminate the Nationwide License Agreement under specified circumstances if we pursue litigation against Nationwide.

Arrowhead Pharmaceuticals, Inc.

On November 25, 2024, we and Arrowhead Pharmaceuticals, Inc. ("Arrowhead") entered into an Exclusive License and Collaboration Agreement (the "Arrowhead Collaboration Agreement") pursuant to which Arrowhead granted us an exclusive license under certain of Arrowhead's intellectual property rights to develop, manufacture, commercialize, and otherwise exploit the lead candidate (and all backup candidates) for four clinical programs (the "Arrowhead Clinical Programs") and three pre-clinical programs (the "Arrowhead Pre-Clinical Programs"). The Arrowhead Clinical Programs are for targeted siRNA therapies directed to (a) DUX4 for the treatment of facioscapulohumeral muscular dystrophy, (b) DMPK for the treatment of type 1 myotonic dystrophy, (c) ATXN2 for the treatment of ataxias, and (d) MMP7 for the treatment of idiopathic pulmonary fibrosis. The pre-clinical programs are for targeted siRNA therapies directed to (a) ATXN1 for the treatment of ataxias, (b) ATXN3 for the treatment of ataxias, and (c) HTT for the treatment of Huntington's Disease. We will also collaborate on the discovery and development of compounds that are directed to six targets to be selected by us during the term (each an "Arrowhead Discovery Program," and together with the Arrowhead Clinical Programs and Pre-Clinical Programs, the "Arrowhead Programs"). The selection of targets will be subject to certain restrictions set forth in the Arrowhead Collaboration Agreement. Subject to certain restrictions set forth in the Arrowhead Collaboration Agreement, if an Arrowhead Discovery Program target is deemed futile, then we will have the right to substitute any such target up to two times.

Exclusivity

Except for its performance of activities under the Arrowhead Collaboration Agreement, Arrowhead may not perform any development or commercialization activities with respect to any compounds or products (a) directed to any target that is the subject of activities under the Arrowhead Collaboration Agreement until the target for such Arrowhead Program has been terminated, (b) for the treatment of spinocerebellar ataxias until the ATXN1 Program, ATXN2 Program, and ATXN3 Program have all been terminated, or (c) directed to a list of reserved skeletal muscle targets for five years, provided that two of the reserved skeletal muscles will be subject to an additional two years of exclusivity.

Development, Manufacturing, and Commercialization

Arrowhead will conduct development activities with respect to the Arrowhead Programs under the Arrowhead Collaboration Agreement pursuant to agreed-upon development plans. At pre-determined transition points for each Arrowhead Program, Arrowhead will transfer development responsibility to us. We will then perform all development activities in furtherance of obtaining and maintaining regulatory approvals for licensed products throughout the world. We will reimburse Arrowhead for certain pre-determined development activities for the Arrowhead Clinical Programs. Each party is responsible for the costs and expenses of other development activities under the Arrowhead Collaboration Agreement.

Arrowhead will complete all manufacturing activities necessary for Arrowhead's development activities for each Arrowhead Program. Arrowhead will also provide clinical supply of licensed compounds and licensed products for all Arrowhead Programs under the Arrowhead Collaboration Agreement and commercial supply of licensed compounds and licensed products for the Arrowhead Clinical Programs. The parties will determine at a later date whether Arrowhead will provide commercial supply of licensed compounds and licensed products for the Arrowhead Preclinical Programs and Arrowhead Discovery Programs. Upon the occurrence of certain conditions, Arrowhead will transfer control of manufacturing and supply to us.

We will have the sole right to commercialize licensed products throughout the world.

Governance

The exploitation of licensed compounds and licensed products will be governed by a series of committees established to facilitate transition and collaboration between the parties with respect to development and manufacturing of such products.

Financial Terms

At closing, on February 7, 2025, we paid Arrowhead an up-front payment of $500.0 million in cash. Arrowhead has the potential to receive $300.0 million in near-term payments associated with the continued enrollment of certain cohorts of a Phase 1/2 study. Additionally, Arrowhead is eligible to receive up to $250.0 million in annual fees and, for each of the Programs, Arrowhead is eligible to receive development milestone payments between $110.0 million and $180.0 million per Arrowhead Program and sales milestone payments between $500.0 million and $700.0 million per Arrowhead Program from us.

In addition, the Arrowhead Collaboration Agreement provides that we will pay Arrowhead tiered royalties on annual net sales of all licensed products for a given Arrowhead Program, up to the low double digits.

Term; Termination

Unless earlier terminated as described below, the Arrowhead Collaboration Agreement will continue on a licensed product-by-licensed product and country-by-country basis, until the expiration of the royalty term for such licensed product in such country. The Arrowhead Collaboration Agreement includes a customary royalty term.

Either party may terminate the Arrowhead Collaboration Agreement for the other party's material breach if such breach is not cured within a specified cure period.

We may terminate the Arrowhead Collaboration Agreement for convenience, in its entirety, on an Arrowhead Program-by-Arrowhead Program and region-by-region basis prior to the first regulatory approval for a licensed product that is the subject of such Arrowhead Program, or on a licensed product-by-licensed product and region-by-region basis after the first regulatory approval for a licensed product that is the subject of such Arrowhead Program. If there is a clinical trial failure of the ongoing clinical trial for ARO-DM1, then, at our election, we may terminate the Arrowhead Collaboration Agreement with respect to either the DM1 Program or ARO-DM1.

Equity Investment

In connection with the Arrowhead Collaboration Agreement, Sarepta Therapeutics Investments, Inc., a wholly owned subsidiary of Sarepta ("Sarepta Investments"), purchased 11,926,301 shares of common stock, par value $0.001 per share, of Arrowhead (the "Arrowhead Shares"), in a private placement transaction, for an aggregate purchase price of $325.0 million on February 7, 2025.

Patents and Proprietary Rights

Our success depends in part upon our ability to obtain and maintain exclusivity for our products, product candidates and platform technologies. We typically rely on a combination of patent protection and regulatory exclusivity to maintain exclusivity for our products and product candidates, whereas exclusivity for our platform technologies is generally based on patent protection and trade secret protection. In addition to patent protection, regulatory exclusivity, and trade secret protection, we protect our products, product candidates and platform technologies with copyrights, trademarks, and contractual protections.

We actively seek patent protection for our product candidates and certain of our proprietary technologies by filing patent applications in the U.S. and other countries as appropriate. These patent applications are directed to various inventions, including, but not limited to, active ingredients, pharmaceutical formulations, methods of use, and manufacturing methods. In addition, we actively acquire exclusive rights to third party patents and patent applications to protect our in-licensed product candidates and corresponding platform technologies.

We do not have patents or patent applications in every jurisdiction where there is a potential commercial market for our product candidates. For each of our programs, our decision to seek patent protection in specific foreign markets, in addition to the U.S., is based on many factors, including:

- our available resources;
- the number and types of patents already filed or pending;
- the likelihood of success of the product candidate;
- the size of the commercial market;
- the presence of a potential competitor in the market; and
- whether the legal authorities in the market effectively enforce patent rights.

We continually evaluate our patent portfolio and patent strategy and believe our owned and licensed patents and patent applications provide us with a competitive advantage; however, if markets where we do not have patents or patent applications become commercially important, our business may be adversely affected. A discussion of certain risks and uncertainties that may affect our freedom to operate, patent position, regulatory exclusivities and other proprietary rights is set forth in Item 1A. Risk Factors included in this report, and a discussion of legal proceedings related to the key patents protecting our products and product candidates are set forth below in the footnotes to the tables in this section.

Certain of our product candidates are in therapeutic areas that have been the subject of many years of extensive research and development by academic organizations and third parties who may control patents or other intellectual property that they might assert against us, should one or more of our product candidates in these therapeutic areas succeed in obtaining regulatory approval and thereafter be commercialized. We continually evaluate the intellectual property rights of others in these areas in order to determine whether a claim of infringement may be made by others against us. Should we determine that a third party has intellectual property rights that could impact our ability to freely market a compound, we consider a number of factors in determining how best to prepare for the commercialization of any such product candidate. In making this determination we consider, among other things, the stage of development of our product candidate, the anticipated date of first regulatory approval, whether we believe the intellectual property rights of others are valid, whether we believe we infringe the intellectual property rights of others, whether a license is available upon commercially reasonable terms, whether we will seek to challenge the intellectual property rights of others, the term of the rights, and the likelihood of and liability resulting from an adverse outcome should we be found to infringe the intellectual property rights of others.

Currently, U.S. patents, as well as most foreign patents, are generally effective for 20 years from the date the earliest regular application was filed. In some countries, the patent term may be extended to recapture a portion of the term lost during regulatory review of the claimed therapeutic. For example, in the U.S., under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly known as the Hatch-Waxman Act, a patent that covers an FDA-approved drug may be eligible for patent term extension (for up to five years, but not beyond a total of 14 years from the date of product approval) as compensation for patent term lost during the FDA regulatory review process. In the U.S., only one patent may be extended for any product based on FDA delay. In addition to patent term extension, patents in the U.S. may be granted additional term due to delays at the USPTO during prosecution of a patent application. We actively strive to maximize the potential for patent protection for our products and product candidates in accordance with the law.

Key Patents & Regulatory Exclusivities

Our products, product candidates and our technologies are primarily protected by composition of matter and methods of use patents and patent applications. Below we provide a summary of granted US and European composition of matter or method of use patents that relate to our marketed products and that we either: (1) solely or with another party own or control, or (2) exclusively license. To the extent the product indicated above the tables that immediately follow the name of such product is covered by a patent that is licensed to Sarepta, we may owe milestones and/or royalties to the indicated licensor in connection with the development and/or commercial sale of the product.

The various types of regulatory exclusivity for which our products have been granted and our product candidates are anticipated to be eligible to receive are shown below, and generally discussed, under ‘Government Regulation’ – ‘Data and Market Exclusivities’ and ‘Orphan Drug Designation and Exclusivity’.

Delandistrogene moxeparvovec-rokl

Patent Number	Country/Region	Patent Type	Expiration Date	Owner/Licensor
11,723,986	United States	Composition of Matter	September 26, 2037	Nationwide
EP 3 442 602 B1	Europe	Composition of Matter & Methods of Use	April 14, 2037	Nationwide
EP 3 596 222 B1	Europe	Composition of Matter & Methods of Use	March 16, 2038	Nationwide

In connection with its FDA approval on June 22, 2023, the FDA granted ELEVIDYS (delandistrogene moxeparvovec-rokl) data exclusivity until June 22, 2035, and Orphan Drug Exclusivity until June 22, 2030.

Eteplirsen

Patent Number	Country/Region	Patent Type	Expiration Date	Owner/Licensor
U.S. RE47,751[1]	United States	Methods of Use	June 28, 2025	UWA
U.S. 9,018,368	United States	Composition of Matter	June 28, 2025	UWA
US 10,781,451	United States	Composition of Matter	June 28, 2025	UWA
U.S. RE48,468[2]	United States	Methods of Use	October 27, 2028	BioMarin/AZL

U.S. RE47,769[3]	United States	Composition of Matter	February 2, 2029	UWA
U.S. 9,506,058	United States	Methods of Use	March 14, 2034	Sarepta
U.S. 10,364,431	United States	Methods of Use	March 14, 2034	Sarepta
U.S. 10,337,003	United States	Methods of Use	March 14, 2034	Sarepta
EP 1 766 010 B1	Europe	Composition of Matter & Methods of Use	June 28, 2025	UWA

1. Reissue of U.S. 8,486,907, which previously was involved in U.S. Patent Interference No. 106,013 and ordered to be cancelled pursuant to Judgment dated September 29, 2015 (Decision dated December 29, 2015 denied our (UWA) Request for Rehearing. Appeal by us (UWA) to the Court of Appeals for the Federal Circuit (Case Nos. 2016-1937, 2016-2086 (consolidated)) voluntarily dismissed July 27, 2017.)
2. Reissue of U.S. 9,243,245.
3. Reissue of U.S. 7,807,816, which previously was involved in U.S. Patent Interference No. 106,008 (Judgment dated September 20, 2016 ordered cancellation of all claims of U.S. Application No. 13/550,210 to BioMarin (AZL). Appeal by BioMarin (AZL) to the Court of Appeals for the Federal Circuit (Case No. 2017-1078) voluntarily dismissed July 27, 2017.)

Golodirsen

Patent Number	Country/Region	Patent Type	Expiration Date	Owner/Licensor
U.S. RE47,691[1]	United States	Composition of Matter	June 28, 2028	UWA
U.S. 9,024,007	United States	Composition of Matter	June 28, 2025	UWA
U.S. 9,994,851[2]	United States	Composition of Matter	June 28, 2025	UWA
U.S. 10,266,827[2]	United States	Methods of Use	June 28, 2025	UWA
U.S. 10,227,590[2]	United States	Composition of Matter	June 28, 2025	UWA
U.S. 10,421,966	United States	Composition of Matter	June 28, 2025	UWA
U.S. 10,968,450	United States	Composition of Matter	June 28, 2025	UWA
U.S. 10,995,337	United States	Composition of Matter & Methods of Use	June 28, 2025	UWA
EP 2 970 964 B1	Europe	Composition of Matter	March 14, 2034	Sarepta

1. Reissue of U.S. 8,455,636, which previously was involved in U.S. Patent Interference No. 106,007. (Judgment dated April 29, 2016 ordered cancellation of (i) all claims, except claim 77, of U.S. Application No. 11/233,495 to BioMarin (AZL); and (ii) U.S. 8,455,636 to us (UWA). Appeal by BioMarin (AZL) to the Court of Appeals for the Federal Circuit (Case No. 2016-2262) voluntarily dismissed July 27, 2017.)
2. Involved in *Nippon Shinyaku Co., Ltd. v. Sarepta Therapeutics, Inc.*, C.A. No. 21-1015 (LPS) (D. Del. 2021).

In connection with its FDA approval on December 12, 2019, the FDA granted VYONDYS 53 (golodirsen) Orphan Drug Exclusivity until December 12, 2026.

Casimersen

Patent Number	Country/Region	Patent Type	Expiration Date**	Owner/Licensor
U.S. 9,447,415	United States	Composition of Matter	June 28, 2025	UWA
U.S. RE48,960[1]	United States	Compositions of Matter & Methods of Use	June 28, 2025	UWA
U.S. 9,228,187	United States	Composition of Matter	November 12, 2030	UWA
U.S. 9,758,783	United States	Methods of Use	November 12, 2030	UWA
U.S. 10,287,586	United States	Composition of Matter	November 12, 2030	UWA
U.S. 10,781,450	United States	Methods of Use	November 12, 2030	UWA

EP 2 499 249 B1	Europe	Composition of Matter & Methods of Use	November 12, 2030	UWA

[1.] Reissue of U.S. 8,524,880.

In connection with its FDA approval on February 25, 2021, the FDA granted AMONDYS 45 (casimersen) NCE exclusivity until February 25, 2026, and Orphan Drug Exclusivity until February 25, 2028.

In addition to the foregoing composition of matter and method of use patents, we have rights to patent applications in the U.S. and in major foreign markets that, if granted, would provide additional protection for our products covered therein. These patents, and patent applications, if granted, would expire at various future dates and protection may be further extended by patent term extension, patent term adjustment, supplemental protection certificate or pediatric extensions that may be available.

Trademarks

Our trademarks are important to us and are generally filed to protect our corporate brand, our products and platform technologies. We typically file trademark applications and pursue their registration in the U.S., Europe and other markets in which we anticipate using such trademarks. We are the owner of multiple federal trademark registrations in the U.S. including, but not limited to, Sarepta, Sarepta Therapeutics, the double-helix logo, ELEVIDYS, EXONDYS, EXONDYS 51, the EXONDYS 51 Logo, VYONDYS, VYONDYS 53, the VYONDYS 53 Logo, AMONDYS, AMONDYS 45, and the AMONDYS 45 Logo. In addition, we have multiple pending trademark applications and registrations in the U.S. and in major foreign markets. Trademark protection varies in accordance with local law, and continues in some countries as long as the trademark is used and in other countries as long as the trademark is registered. Trademark registrations generally are for fixed but renewable terms.

Government Regulation

The research, development, testing, manufacturing, labeling, advertising, promotion, distribution, packaging, storage, exportation and marketing of our products are subject to extensive regulation by governmental authorities in the U.S. and in other countries. In the U.S., the FDA, under the Federal Food, Drug and Cosmetic Act, the Public Health Service Act, and their implementing regulations, regulates pharmaceutical products. Failure to comply with applicable U.S. requirements may subject us to administrative or judicial sanctions, such as FDA refusal to approve pending marketing applications, withdrawal of approval of approved products, warning letters, untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, civil penalties and/or criminal prosecution.

U.S. Drug Approval Process

To obtain FDA approval of a product candidate, we must, among other things, submit clinical data providing substantial evidence of safety and efficacy of the product candidate for its intended use, as well as detailed information on product composition, its manufacture and controls and proposed labeling. The testing and collection of data and the preparation of necessary applications are expensive and time-consuming. The FDA may not act quickly or favorably in reviewing these applications, and we may encounter significant difficulties or costs in our efforts to obtain FDA approvals that could delay or preclude us from marketing our products.

The steps required before a drug may be approved for marketing in the U.S. generally include the following:

- completion of pre-clinical laboratory tests and animal toxicity testing, including studies conducted in accordance with good laboratory practice requirements;
- submission and approval of an investigational new drug (“IND”) for conducting human clinical testing to the FDA;
- approval by an Institutional Review Board (“IRB”) or independent ethics committee at each clinical trial site before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with good clinical practice (“GCP”) requirements and other clinical trial-related regulations to establish the safety and efficacy of the drug product for each indication;
- submission of a complete and compliant marketing application containing chemistry, manufacturing and control information for the drug substance and drug product, reports of nonclinical and clinical trials, product labeling and administrative information;

- satisfactory completion of an FDA inspection of the commercial manufacturing facilities at which the drug substance and drug product are made to assess compliance with cGMP;
- satisfactory FDA audit of the clinical trial site(s) that generated the pivotal safety and efficacy data included in the marketing application and also potentially the nonclinical trial site(s) in the form of pre-approval inspections; and
- FDA review and approval of the marketing application.

Pre-clinical trials may include laboratory evaluations of the product chemistry, pharmacology, toxicity and formulation, as well as animal studies to assess the pharmacokinetics, metabolism, bio-distribution, elimination and toxicity of the product candidate. The conduct of the pre-clinical tests and formulation of the compounds for testing must comply with federal regulations and requirements. The results of the pre-clinical trials, manufacturing information, analytical data and a proposed first in human clinical trial protocol are submitted to the FDA as part of the IND, which must become effective before clinical trials may be initiated. The IND will become effective 30 days after receipt by the FDA, unless the FDA raises concerns or questions about the supportive data, or the study design, particularly regarding potential safety issues with conducting the clinical trial as described in the protocol. In this situation, the trials are placed on clinical hold and the IND sponsor must resolve any outstanding FDA concerns before clinical trials can proceed.

Clinical trials involve the administration of the product candidate to healthy volunteers or patient participants under the supervision of a qualified principal investigator. Clinical trials are conducted under protocols detailing the objectives of the study, the administration of the investigational product, subject selection and exclusion criteria, study procedures, parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol must be submitted to the FDA as a submission to the IND. Clinical trials must be conducted and monitored in accordance with the FDA's GCP requirements and federal and state laws and regulations protecting study subjects. Further, each clinical trial must be reviewed and approved by the IRB at or servicing each institution in which the clinical trial will be conducted. Both the FDA and IRB can temporarily or permanently halt a clinical trial at any time, or impose other sanctions or conditions, if it believes that the clinical trial is not being conducted in accordance with FDA requirements, GCP or IRB requirements or that it presents an unacceptable risk to the clinical trial subjects.

Clinical trials typically are conducted in three sequential drug development phases (Phases 1, 2 and 3) prior to approval, and a portion of these phases may overlap. A fourth post-approval phase (Phase 4) may include additional clinical trials. A general description of clinical trials conducted in each phase of development is provided below. However, the number of study subjects involved in each phase of drug development for rare diseases can be significantly less than typically expected for more common diseases with larger patient populations:

- Phase 1. Phase 1 clinical trials involve the initial introduction of the drug into human subjects. These studies are usually designed to determine the safety of single and multiple doses of the compound and determine any dose limiting toxicities or intolerance, as well as the metabolism and pharmacokinetics of the drug in humans. Phase 1 studies usually involve fewer than 100 subjects and are conducted in healthy adult volunteers, unless it is unethical to administer the study drug to healthy volunteers, in which case they are tested in patients.
- Phase 2. Phase 2 clinical trials are usually conducted in a limited patient population to evaluate the safety and efficacy of the drug for a specific indication to determine optimal dosage and to identify possible adverse effects and safety risks. Phase 2 studies usually involve patients with the disease under investigation and may vary in size from several dozen to several hundred.
- Phase 3. Larger Phase 3 clinical trials are conducted to confirm clinical efficacy, dosage and safety in the intended patient population, which may involve geographically dispersed clinical trial sites. Generally, two adequate and well-controlled Phase 3 clinical trials which establish the safety and efficacy of the drug for a specific indication are required for approval of a marketing application. Phase 3 studies usually include several hundred to several thousand patients for larger, non-orphan drug indications/diseases. However, clinical trials for rare or orphan diseases generally have fewer patients due to their lower prevalence. For these orphan diseases, a company may also try to demonstrate efficacy and safety by comparing treated patients in clinical trials to untreated patients participating in placebo-controlled clinical trials or to observational natural history studies.
- Phase 4. Phase 4 trials are clinical trials conducted after the FDA has approved a product for marketing. Typically, there are two forms of Phase 4 trials: those that are conducted to fulfill mandatory conditions of product approval and those that are voluntarily conducted to gain additional experience from the treatment of patients in the intended therapeutic indication.

A company seeking marketing approval for a new drug in the U.S. must submit the results of the pre-clinical and clinical trials to the FDA in the form of a marketing application, together with, among other things, detailed information on the manufacture and composition of the product candidate and proposed labeling, including payment of a user fee for FDA review of the application. The user fee is waived for an application for a product intended to treat an Orphan Indication. To support marketing approval, the data

submitted must be sufficient in quality and quantity to establish the safety and efficacy of the investigational drug, or the safety, purity and potency of the investigational biologic, to the satisfaction of the FDA. FDA approval of a marketing application must be obtained before a drug or biologic may be marketed in the U.S.

The FDA assesses all submitted marketing applications for completeness before it accepts them for filing, a decision which must be made within 60 days of receipt. In some cases, the FDA may request additional information in a resubmitted application before accepting a marketing application for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the marketing application. Applications receive either standard or priority review. Under the current goals mandated under the Prescription Drug User Fee Act (the "PDUFA"), the FDA has ten months in which to complete its initial review of a standard marketing application and respond to the applicant, and six months for a priority marketing application, though the FDA does not always meet its PDUFA goal dates. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the marketing application sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date. The FDA may refer an application to an advisory committee for review, evaluation and issuance of a non-binding recommendation as to whether the application should be approved. If the FDA's evaluations of the marketing application and the clinical and manufacturing procedures and facilities are favorable, the FDA may issue an approval letter, authorizing commercial marketing of the drug. If the FDA finds deficiencies in the marketing application, it may issue a complete response letter ("CRL"), which defines the conditions that must be met in order to secure final approval of the marketing application. Sponsors that receive a CRL may submit to the FDA information that represents a complete response to the issues identified by the FDA. Resubmissions by the marketing application sponsor in response to a CRL trigger new review periods of varying length (typically two to six months) based on the content of the resubmission.

Even if the FDA approves a product, depending on the specific risk(s) to be addressed, the FDA may limit the approved indications for use of the product; require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess a product's safety or efficacy after approval, require testing and surveillance programs to monitor the product after commercialization; or impose other conditions, including distribution and use restrictions or other risk management mechanisms under a risk evaluation and mitigation strategy, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-marketing studies or surveillance programs. After approval, some types of changes to the approved product, such as adding new indications, manufacturing changes, and additional labeling claims, are subject to further testing requirements and FDA review and approval.

A sponsor may also seek designation of its drug candidates under programs designed to accelerate the FDA's review and potential approval of marketing applications. For instance, a sponsor may seek FDA designation of a drug candidate as a "fast track product," a "breakthrough therapy product," or a "Regenerative Medicine Advanced Therapy ("RMAT")" designated product, or may seek approval through the accelerated approval pathway or under priority review.

- *Fast Track Designation:* Fast track products are those products intended for the treatment of a serious or life-threatening disease or condition and which demonstrate the potential to address unmet medical needs for such disease or condition. If fast track designation is obtained, the FDA may initiate early and frequent communication and begin reviewing sections of a marketing application before the application is complete. This "rolling review" is available if the applicant provides, and the FDA approves, a schedule for the remaining information.
- *Breakthrough Therapy Designation*: Breakthrough therapy designation is focused on expediting the development and review process and by itself does not create an alternate ground for product approval. A sponsor may seek FDA designation of a drug candidate as a breakthrough therapy if the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The FDA issued guidance entitled "Expedited Programs for Serious Conditions—Drugs and Biologics" in May 2014.
- *RMAT Designation*: RMAT designation may be granted to drug products that meet the statutory definition of RMAT; are intended to treat, modify, reverse, or cure a serious condition; and for which preliminary clinical evidence indicates that the RMAT has the potential to address unmet clinical needs for such condition. The statutory definition of an RMAT includes therapies such as our gene therapy product candidates.
- *Accelerated Approval*: the FDA may also approve products through the accelerated approval pathway, which is aimed at expediting review of drugs that treat serious conditions and provide a meaningful advantage over available therapies. Accelerated approval is based on demonstrated effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality ("IMM") that is reasonably likely to predict an effect on IMM or other clinical benefit (i.e., an intermediate clinical endpoint). Approvals of this kind typically include requirements for appropriate post-approval Phase 4 clinical trials to confirm clinical benefit.

The Food and Drug Omnibus Reform Act of 2022 ("FDORA") signed by former President Biden on December 29, 2022 as part of the Consolidated Appropriations Act, 2023 (H.R. 2617) included numerous reforms to the accelerated approval process including, among other things, (i) enabling the FDA to require, as appropriate, that a post-approval study be underway prior to granting accelerated approval; and (ii) expanding the expedited withdrawal procedures available to the FDA for revoking accelerated approvals if a sponsor fails to conduct any required post-approval study with due diligence the FDA has issued guidance documents clarifying each of these reforms in January 2025 and December 2024, respectively.

- *Priority Review*: If a drug candidate demonstrates a significant benefit over existing therapy, it may be eligible for priority review, which means it will be reviewed within a six-month timeframe from the date a complete marketing application is accepted for filing.

We cannot be sure that any of our drug candidates will qualify for any of these expedited development, review and approval programs, or that, if a drug does qualify, that the product candidates will be approved, will be accepted as part of any such program or that the review time will be shorter than a standard review.

Holders of an approved marketing application are required to:

- report serious adverse drug reactions to the FDA;
- submit annual and periodic reports summarizing product information and safety data;
- comply with requirements concerning advertising and promotional labeling;
- continue to have quality control and manufacturing procedures conform to cGMP after approval; and
- conduct any post-marketing study designated as a required condition of the marketing application approval.

The FDA periodically inspects the sponsor's records related to safety reporting and/or manufacturing; this latter effort includes assessment of compliance with cGMP. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved marketing application, including withdrawal of the product from the market.

Foreign Regulatory Requirements

In 2018, the Committee for Medicinal Products for Human Use ("CHMP") within the EMA confirmed its negative opinion for eteplirsen, and the European Commission ("EC") adopted an implementing decision to ratify the CHMP opinion to refuse marketing authorization.

As of the date of this Annual Report, EXONDYS 51, has been approved for sale and marketing in the U.S., Israel, Libya, Georgia and Kuwait, and AMONDYS 45 and VYONDYS 53 have been approved for sale and marketing in the U.S., Libya and Kuwait. We have received approval for sale and marketing for ELEVIDYS in the U.S., and our strategic partner Roche has received approvals in certain other countries.

Thus, in addition to regulations in the U.S., our business is subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products. Irrespective of whether it concerns an FDA approved or investigational drug, the commencement of clinical trials and the subsequent marketing of a drug product in foreign countries are subject to preliminary approvals from the corresponding regulatory authorities of such countries. For example, the conduct of clinical trials in the EU is governed by the Clinical Trials Regulation (EU) No 536/2014 (the "CTR") and the principles and guidelines on GCP.

In April 2014, the EU adopted the CTR, which is now in application. The CTR requires a clinical trial sponsor to obtain a clinical trial authorization ("CTA") from the national competent authority ("NCA") of each EU member state in which the clinical trial is to be conducted. Furthermore, the sponsor can only start a clinical trial at a specific study site after the local research ethics committee has issued a favorable opinion.

Subject to the transition arrangement referenced below, a sponsor must submit a single application for a CTA, through a centralized EU clinical trials portal, the Clinical Trials Information System ("CTIS"). One NCA (the reporting EU member state selected by the sponsor) takes the lead in validating and evaluating the application, as well as consulting and coordinating with the other concerned member states in which the clinical trial is to be conducted. If an application is rejected, it may be amended and resubmitted through CTIS. A concerned member state may in limited circumstances declare an "opt-out" from an approval and prevent the clinical trial from being conducted in that member state.

The CTR foresees a three-year transition period. As of January 31, 2023, all new CTAs had to be submitted via CTIS and made pursuant to the CTR. By January 31, 2025, all clinical trials that are still ongoing and that were authorized under the Directive 2001/20/EC (which was replaced by the CTR), must be transitioned to the new regime.

In order to obtain marketing authorization for a medicinal product in the EU, applicants are required to submit a marketing authorization ("MA") application ("MAA") to either (a) the NCAs of the EU member states of interest (through the decentralized, mutual recognition, or national procedures) if the medicinal product does not fall within the mandatory scope of the centralized procedure or (b) the EMA (through the centralized authorization procedure). Irrespective of the procedure, applicants are required to demonstrate the quality, safety and efficacy of the medicinal product in the application for MA, which implies the requirement to conduct human clinical trials to generate the necessary clinical data.

Regulation (EC) No 726/2004 of the European Parliament and of the Council lays down the rules applicable to the centralized procedure for the authorization of medicinal products. The centralized procedure allows pharmaceutical companies to submit a single MAA to the EMA, which, if successful, results in a single MA to market the medicinal product throughout the entire EU and Iceland, Liechtenstein and Norway (collectively, the "EEA"). Approval via the centralized procedure is a two-step process whereby the CHMP first evaluates the MAA and issues an opinion on whether the medicinal product may be authorized or not (step 1). The CHMP opinion is subsequently sent to the EC, which takes a legally binding decision to grant a MA (step 2). The MA is valid throughout the EEA and is automatically recognized in Iceland, Lichtenstein and Norway. This allows the MA holder to market the medicine and make it available throughout the entire EEA. The timeframe for the first step of the centralized procedure (evaluation by the CHMP) opinion is 210 days from receipt of a valid application. However, the actual time needed to complete this first step is generally longer than the 210 days, since procedural clock stops are required in order for the applicant to respond to additional requests for information by the CHMP. Following a positive CHMP opinion, the EC has generally 67 days to issue its decision to grant the MA or not.

Accelerated evaluation of the MAA under the centralized procedure is possible in exceptional cases, following a justified request from the applicant, when a medicinal product is of major interest for public health, particularly from the point of view of therapeutic innovation. The CHMP determines what constitutes a major public interest on a case-by-case basis. If the applicant provides sufficient justification for an accelerated assessment, the CHMP can reduce the timeframe for review of a MAA to 150 days, excluding a limited procedural clock-stop. The timeframe for the EC to issue its decision remains unaltered.

In relation to the EEA, Article 3 of Regulation (EC) No 726/2004 defines in which cases the centralized application procedure must (mandatory scope) or may (optional scope) be followed. The centralized procedure is mandatory for certain types of medicinal products, including those developed using a biotechnological process (such as recombinant DNA technology, controlled expression of genes coding for biologically active proteins in prokaryotes and eukaryotes, including transformed mammalian cells, hybridoma and monoclonal antibody methods), orphan medicinal products, advanced therapy medicinal products (gene-therapy, somatic cell-therapy or tissue-engineered medicines) and products containing a new active substance indicated for the treatment of HIV/AIDS, cancer, diabetes, auto-immune and other immune dysfunctions, viral diseases and neurodegenerative diseases biotechnology medicinal products, orphan medicinal products, advanced-therapy medicinal products. For medicinal products that do not fall under any of the aforementioned categories, a submission via the centralized procedure is possible, provided that it concerns (i) a new active substance or (ii) product that can demonstrate a significant therapeutic, scientific or technical innovation and for which approval would be in the interest of public health. Given the foregoing, our portfolio of innovative orphan products for neurodegenerative diseases is subject to the mandatory centralized procedure.

Similar to the U.S., MA holders and manufacturers of medicinal products are subject to comprehensive regulatory oversight by the EMA and/or the NCA of the EU member states. This oversight applies both before and after the granting of manufacturing and MAs. It includes compliance with EU GMP and GDP rules in relation to such activities as distribution, importing and exporting of medicinal products, rules governing conduct of pharmacovigilance (including good pharmacovigilance practices) and requirements governing advertising, promotion and sale of medicinal products.

Failure to comply with the EU member state laws implementing the EU Community Code on medicinal products, and EU rules governing the promotion of medicinal products, interactions with physicians, misleading and comparative advertising and unfair commercial practices, with the EU Member State laws that apply to the promotion of medicinal products, statutory health insurance, bribery and anti-corruption or with other applicable regulatory requirements can result in enforcement action by the relevant EU Member State authorities. This may include any of the following sanctions: fines, imprisonment, orders forfeiting products or prohibiting or suspending their supply to the market, orders to suspend, vary, or withdraw the marketing authorization or requiring the manufacturer to issue public warnings, or to conduct a product recall.

The approval process in other countries outside the U.S. and the EU varies from country to country, and the time may be longer or shorter than that required for the FDA approval. In addition, the requirements governing the conduct of clinical trials,

product licensing, pricing and reimbursement for market access vary greatly from country to country. In all cases, clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

Data and Market Exclusivities

In addition to patent exclusivities, the FDA and certain other foreign health authorities may grant data or market exclusivity for a newly approved chemical entity or biologic, which runs in parallel to any patent protection. Regulatory data protection or exclusivity prevents a potential generic competitor from relying on clinical trial data generated by the sponsor when establishing the safety and efficacy of its competing product. Market exclusivity prohibits any marketing of the same drug for the same indication.

In the U.S., the FDA will generally grant a NCE that is the subject of an NDA with five years of regulatory data exclusivity, during which time no applications to the FDA for competitor products may be submitted. A competitor, however, may file an application seeking approval of a generic drug four years from the date of approval of the innovative product if it is accompanied by a certification of patent invalidity or noninfringement. The FDA will also grant three years of exclusivity for an NDA for a product that contains an active moiety that has already been approved or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application (for example, new indications, dosages or strengths of an existing drug.) This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving abbreviated new drug applications ("ANDAs") for drugs containing the original active agent for other conditions of use. For a newly approved biologic that is the subject of a biologic license application ("BLA"), the FDA will generally grant 12 years of market exclusivity, during which time a competitor may not market the same drug for the same indication.

In addition, the FDA may provide six months of pediatric exclusivity to a sponsor of a marketing application if the sponsor conducted a pediatric study or studies of a product. This process is applied to products developed for adult use and is initiated by the FDA as a written request for pediatric studies that applies to a sponsor's product. If the sponsor conducts qualifying studies and the studies are accepted by the FDA, then an additional six months of pediatric exclusivity will be added to previously granted exclusivity, such as orphan drug exclusivity and NCE exclusivity, as well as certain patent-based exclusivities.

In relation to the EEA, innovative medicinal products which have been authorized on the basis of a complete independent data package consisting of quality, preclinical testing results and clinical trial data, benefit from an eight-year period of data exclusivity and a ten-year period of marketing protection/exclusivity. During the data exclusivity period, applicants for approval of generics of these innovative products cannot reference or rely upon data contained in the MA dossier submitted for the innovative medicinal product. During the marketing protection period, even if the generic product is approved, it cannot be placed on the market until the full ten-year period of market protection has elapsed from the initial authorization of the reference medicinal product. The marketing protection period can be extended to a maximum of 11 years if, during the first eight years of those ten years, the MA holder for the innovative product obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies.

In the EEA, all applications for MA for new medicines must include the results of studies as described in an agreed pediatric investigation plan ("PIP") aimed at ensuring that the necessary data are obtained through studies in children, unless the medicine is exempt because of a deferral or waiver. PIPs are agreed with the EMA's Pediatric Committee. The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the drug for which MA is being sought. Deferrals allow an applicant to delay development of the medicine in children until, for instance, there is enough information to demonstrate its effectiveness and safety in adults. Waivers, on the other hand, may be granted when the development of a medicine in children is not needed or is not appropriate, such as for diseases that only affect the adult population or when the product does not represent a significant therapeutic benefit over existing treatments for pediatric patients. Products that are granted a MA with the results of the pediatric clinical trials conducted in accordance with the PIP (even where such results are negative) are eligible for six months' supplementary protection certificate extension (if any is in effect at the time of approval). In the case of orphan medicinal products, a two-year extension of the orphan market exclusivity may be available. This pediatric reward is subject to specific conditions and is not automatically available when data in compliance with the PIP are developed and submitted.

Orphan Drug Designation and Exclusivity

In the U.S., the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the U.S., or more than 200,000 individuals in the U.S. for which there is no reasonable expectation that the cost of developing and making available in the U.S. a drug for this type of disease or condition will be recovered from sales in the U.S. for that drug. An orphan drug designation must be requested before submitting an application for marketing approval, and does not shorten the duration of the regulatory review and approval process. The approval of an orphan designation request does not alter the regulatory requirements and process for obtaining marketing approval. Orphan drug designation generally entitles the

product, once approved, to an orphan drug exclusivity period of seven years, which means the FDA may not grant approval to any other application to market the same chemical or biological product for the same indication for a period of seven years, except in limited circumstances, such as where an alternative product demonstrates clinical superiority to the product with orphan exclusivity.

The FDA has historically taken the position that the scope of orphan exclusivity aligns with the approved indication or use of a product, rather than the disease or condition for which the product received orphan designation. However, on September 30, 2021, the U.S. Court of Appeals for the 11th Circuit issued a decision in *Catalyst Pharms., Inc. v. Becerra* holding that the scope of orphan drug exclusivity must align with the disease or condition for which the product received orphan designation, even if the product's approval was for a narrower use or indication. The FDA announced on January 24, 2023 that despite the Catalyst decision, it will continue to apply its longstanding regulations, which tie the scope of orphan exclusivity to the uses or indications for which the drug is approved, rather than to the designation. Particularly due to the Supreme Court's 2024 decision in *Loper Bright Enterprises v. Raimondo*, which overturned the general judicial practice of deference to Agency's interpretations of ambiguous statutes, the FDA's application of its orphan drug regulations post-Catalyst could be the subject of future legislation or to further challenges in court, and it remains to be seen how this decision affects orphan drug exclusivity going forward. In addition, holders of exclusivity for orphan drugs are expected to assure the availability of sufficient quantities of their orphan drugs to meet the needs of patients. Failure to do so could result in the withdrawal of orphan exclusivity for the drug. Competitors may receive approval of different drugs or biologics for the indications for which a prior approved orphan drug has exclusivity.

Pharmaceutical companies can apply for their product to be designated as an orphan medicinal product; such applications must be submitted prior to submitting a MAA. In the EU, applications for orphan designation are evaluated by the EMA's Committee for Orphan Medicinal Products ("COMP") in accordance with Regulation (EC) No 141/2000. In order to qualify as an orphan medicinal product, the medicinal product must be intended to diagnose, prevent or treat a condition that is life-threatening or chronically debilitating, with a prevalence of no more than 5 in 10,000 people in the EU or for which it is unlikely that the development of the medicine would generate sufficient returns to justify the investment needed for its development. In addition, the sponsor is required to demonstrate that no satisfactory method of diagnosis, prevention or treatment of the condition has been authorized in the EU or, if such method exists, the medicinal product is of significant benefit to those affected by the condition as compared to approved methods. The COMP is required to re-assess the granted orphan designation at the time of MA grant to ensure that it continues to meet the criteria for the designation to be maintained. Otherwise, the orphan designation can be revoked. The benefits of being granted orphan designation are significant, including up to ten years of market exclusivity. During this ten-year period, the EMA may not accept an MAA for a similar medicinal product for the same authorized therapeutic indication as the approved orphan medicinal product. Pursuant to Regulation (EC) 1901/2006 on medicinal products for pediatric use, and as mentioned above, the ten-year orphan market exclusivity can be extended to a maximum period of 12 years upon the satisfactory completion of all the studies of the agreed PIP with the pediatric study results reflected in the summary of product characteristics. We have been granted orphan drug designation for eteplirsen in the EU.

Expanded / Early Access

In certain countries, drug products approved by key competent regulatory agencies, including the FDA, can be accessed by patients before the drug has obtained marketing approval in such country. There are various forms of this access including, but not limited to, the actual purchase of product by the purchaser, which is often times the government for patients, on a named patient basis, and providing the product free of charge on a named patient basis for compassionate use. Each country has its own laws and regulations that apply to these forms of access and the extent and nature of such laws and regulations vary by country. For example, in 2018, the so-called Right to Try Act became law in the U.S. The law, among other things, allows eligible patients to access certain investigational new drug products that have completed at least a Phase I clinical trial and that are undergoing investigation for FDA approval without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a pharmaceutical manufacturer to make its drug products available to such eligible patients as a result of the Right to Try Act.

We established a global EAP for eteplirsen, golodirsen and casimersen in some countries where these products currently have not been approved. The EAP provides a mechanism through which physicians can prescribe our products, within their professional responsibility, to patients who meet pre-specified medical and other criteria and can secure funding.

Other Regulatory Requirements

Environmental Laws

In addition to regulations enforced by the FDA and foreign authorities relating to the clinical development and marketing of products, we are or may become subject to regulation under the Occupational Safety and Health Act, the Toxic Substances Control Act, the Resource Conservation and Recovery Act and other present and potential future foreign, federal, state and local laws and regulations. Our research and development processes involve the controlled use of hazardous materials and chemicals and produce waste products. We are subject to federal, state and local environmental laws and regulations governing the use, manufacture, storage, handling and disposal of hazardous materials and waste products. Although we believe that we are in material compliance with applicable environmental laws that apply to us, we cannot predict whether new regulatory restrictions will be imposed by state or federal regulators and agencies or whether existing laws and regulations will adversely affect us in the future. Compliance with environmental laws is not expected to require significant capital expenditure and has not had, and is not expected to have, a material adverse effect on our operations.

Healthcare Fraud and Abuse Laws

We are subject to various federal, state and local laws targeting fraud and abuse in the healthcare industry, including anti-kickback and false claims laws. Violations of fraud and abuse laws may be punishable by criminal or civil sanctions, including fines and civil monetary penalties, and/or exclusion from federal health care programs (including Medicare and Medicaid). Compliance is challenging. The scope of the federal and the various analogous state anti-kickback, false claims, and similar fraud and abuse laws vary, but is generally broad. Many of the fraud and abuse laws and regulations contain ambiguous requirements or require administrative guidance for implementation. Violations of international fraud and abuse laws could result in similar penalties, including exclusion from participation in health programs outside the U.S. Federal and state authorities are paying increased attention to enforcement of these laws within the pharmaceutical industry, and private individuals have been active in alleging violations of the laws and bringing suits on behalf of the government under the federal False Claims Act ("FCA") as evidenced by numerous significant settlements. Violations of international fraud and abuse laws could result in similar penalties, including exclusion from participation in health programs outside the U.S. Given the scope, complexity and lack of clarity in laws and their implementation, our activities could be subject to scrutiny and the imposition of penalties under the laws. If we were subject to allegations concerning, or were convicted of violating, these laws, our business could be harmed.

The federal Anti-Kickback Statute generally prohibits, among other things, a pharmaceutical manufacturer from directly or indirectly soliciting, offering, receiving, or paying any remuneration in cash or in kind where one purpose is either to induce the referral of an individual for, or the purchase or prescription of, a particular drug that is payable by a federal health care program, including Medicare or Medicaid. A person or entity does not need to have actual knowledge of the statute or a specific intent to violate the statute. A claim arising from a violation of the federal Anti-Kickback Statute also constitutes a false or fraudulent claim for purposes of the FCA. Another healthcare anti-kickback statute prohibits certain payments related to referrals of patients to certain providers (such as clinical laboratories) and applies to services reimbursed by private health plans as well as government health care programs.

Federal and state false claims laws generally prohibit anyone from knowingly and willfully, among other activities, presenting, or causing to be presented for payment to third party payors (including Medicare and Medicaid) claims for drugs or services that are false or fraudulent. Such laws are not always limited to activities involving government programs or payors. For example, a federal healthcare fraud statute prohibits the knowing and willful execution, or attempt to execute, a scheme to defraud a health care benefit program, including private health plans, or obtain, through false or fraudulent pretenses, money or property owned by, or under the custody or control of, such a health care benefit program.

Laws and regulations have also been enacted by the federal government and various states to regulate the sales and marketing practices of pharmaceutical manufacturers. The laws and regulations generally limit financial interactions between manufacturers and health care providers; require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government; and/or require disclosure to the government and/or public of financial interactions (so-called "sunshine laws"). State laws may also require disclosure of pharmaceutical pricing information and marketing expenditures. Manufacturers must also submit information to the FDA on the identity and quantity of drug samples requested and distributed by a manufacturer during each year.

Similar to the Anti-Kickback Statue in the U.S., the provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is also prohibited in the EU. The provision of benefits or advantages to induce or reward improper performance generally is governed by the national anti-bribery laws of EU member states, and the Bribery Act 2010 in the UK. Infringement of these laws could result in substantial fines and imprisonment. Further, Directive 2001/83/EC, which governs medicinal products for human use, further provides that, where

medicinal products are being promoted to persons qualified to prescribe or supply them, no gifts, pecuniary advantages or benefits in kind may be supplied, offered or promised to such persons unless they are inexpensive and relevant to the practice of medicine or pharmacy. Given the broad scope of these laws, our activities could be subject to scrutiny under the laws. If we were subject to allegations concerning, or were convicted of violating, these laws, our business could be harmed.

Data Privacy and Security

We may be subject to privacy and security laws in the various jurisdictions in which we operate, obtain or store personally identifiable information. The legislative and regulatory landscape for privacy and data protection continues to evolve, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Our ongoing efforts to comply with evolving laws and regulations may be costly and require ongoing modifications to our policies, procedures and systems. Failure to comply with laws regarding data protection would expose us to risk of enforcement actions and penalties under such laws. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our business, financial condition, results of operations or prospects.

Within the U.S., there are numerous federal and state laws and regulations related to the privacy and security of personal information. For example, at the federal level, the Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), as amended, and its implementing regulations establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information. While we have determined that we are neither a "covered entity" nor a "business associate" directly subject to HIPAA, many of the U.S. health care providers with which we interact are subject to HIPAA, and we may have assumed obligations related to protecting the privacy of personal information. States are increasingly regulating the privacy and security of personal information. In some states, such as California and Washington, state privacy laws are even more protective than HIPAA. For example, the California Consumer Privacy Act as amended and expanded by the California Privacy Rights Act (together, the "CCPA"), gives California consumers (defined to include all California residents) certain rights, including the right to ask covered companies to disclose copies of personal information collected and delete a consumer's personal information and requires covered companies to provide notice to California consumers regarding their data processing activities. The CCPA places limitations on a covered company's ability to sell personal information and share it for purposes of cross-context behavioral advertising. Similar laws are in operation or have been adopted in eleven other states.

In addition, the processing of personal data relating to EEA citizens or in the context of the activities of an establishment in the EEA is subject to the General Data Protection Regulation (the "GDPR"). The GDPR increases obligations with respect to clinical trials conducted in the EEA, such as in relation to the provision of fair processing notices, responding to data subjects who exercise their rights and reporting certain data breaches to regulators and affected individuals. The GDPR also requires us to enter certain contractual arrangements with third parties that process GDPR-covered personal data on our behalf. The GDPR also increases the scrutiny applied to transfers of personal data from the EEA (including from clinical trial sites in the EEA) to countries that are considered by the EC to lack an adequate level of data protection. Similar rules and requirements are applicable in the UK by virtue of section 3 of the European Union (Withdrawal) Act 2018 and as amended by the Data Protection, Privacy and Electronic Communications (Amendments etc.) (EU Exit) Regulations 2019 (together, the "UK GDPR"). If our or our partners' or service providers' privacy or data security measures fail to comply with the requirements of the GDPR or UK GDPR, we may be subject to litigation, regulatory investigations, enforcement notices requiring us to change the way we use personal data and/or fines of up to EURO 20 million (£17.5 million in the U.K.) or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, as well as claims by affected individuals, negative publicity, reputational harm and a potential loss of business and goodwill.

Pharmaceutical Pricing and Reimbursement

Our revenue depends, in part, upon the extent to which payors provide coverage for our products and the amount that payors, including government authorities or programs, private health insurers and other organizations, reimburse patients and healthcare providers for the cost of our products. Reimbursement coverage policies and inadequate reimbursement may reduce the demand for, or the price purchasers are willing to pay for, our or our partners' products. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided, and reimbursement is adequate to cover a significant portion of the cost of such products.

We have an ongoing dialogue with payors globally with the goal of obtaining broad coverage for our products. To date, payors' policies on coverage for our products have varied widely, including policies that allow broad coverage per the respective product's prescribing information, policies that provide limited coverage and policies that have denied coverage. The majority of payors have policies that provide for case-by-case coverage or restricted coverage.

Third Party Reimbursement and Pricing in the U.S.

Within the U.S., coverage and reimbursement for drug products can differ significantly from payor to payor. One third-party payor's decision to cover a particular drug product does not ensure that other payors will also provide coverage for the drug product. Even if products are covered, third party payors may seek to control utilization of the products through various mechanisms (e.g., requiring a prescriber to obtain prior authorization from a health plan before the product will be covered by the health plan or establishing patient copays and deductibles that encourage use of other products over our products). Coverage of a product by a third-party payor does not mean that reimbursement will be adequate. Third party reimbursement may not be sufficient to enable us to maintain price levels high enough to realize an appropriate return on our investment in product development. Net prices for drugs may be reduced by mandatory discounts or rebates required by government healthcare programs or requested by private payors in exchange for coverage or by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold. Our ability to commercialize our product candidates successfully may be adversely affected by discounts or rebates that we are required to provide in order to ensure coverage of our products and compete in the marketplace.

Significant uncertainty exists as to the coverage and reimbursement status of new drug products. There may be considerable delays in obtaining reimbursement for newly-approved drugs, and coverage may be more limited than the indications for which the drug is approved by the FDA. Third-party payors may also seek, with respect to an approved product, additional clinical evidence, including comparative effectiveness evidence, that goes beyond the data required to obtain marketing approval in order to demonstrate clinical benefits and value relative to other therapies before covering new products. If so, we may be required to conduct additional pharmacoeconomic studies beyond what is required for marketing approval.

We cannot be sure that adequate coverage and reimbursement will be available, or remain available, for any drug that we commercialize. Coverage and reimbursement may impact the demand for, or the price of, our products and any product candidate for which we obtain marketing approval and limits on coverage and reimbursement may adversely affect our ability to successfully commercialize any product candidate for which we obtain marketing approval.

Third Party Reimbursement and Pricing outside the U.S.

We currently have three products approved for marketing outside the U.S. EXONDYS 51 has been approved for marketing in the U.S., Georgia, Israel, Libya and Kuwait, VYONDYS 53 in U.S., Libya and Kuwait, and AMONDYS 45 in the U.S., Libya and Kuwait. We may need to conduct long-term pharmacoeconomic studies in order to demonstrate the cost-effectiveness of our products.

In the EU and certain other territories, price controls and Health Technology Assessments for new, highly priced medicines are expected, and in some cases, mandated. Criteria such as cost-effectiveness, cost per quality-adjusted life year, budget impact, or others, in addition to the clinical benefit, are often required to demonstrate added value or benefit of a drug and vary by country.

EU member states may approve a specific price for a product, by, for example, international reference pricing, or they may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other member states allow companies to fix their own prices for products but monitor and control prescription volumes and issue guidance on prescribing criteria to physicians, having an effect on restricting prescriptions or usage. Recently, many countries in the EU have decided to apply significant discounts to prices of pharmaceuticals and these efforts could continue as countries attempt to manage healthcare expenditures. Political, economic and regulatory developments may further complicate pricing negotiations. Third party reimbursement limits may reduce the demand for our products. The pace of the application process in some countries could also delay commercial product launches. Gaining acceptance of our product pipeline and economically viable reimbursement terms in the EU and other markets will require strong education and awareness efforts around Duchenne as well as strong data supporting its effectiveness and cost-effectiveness. In particular, certain countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies in order to obtain reimbursement or pricing approval. Parallel trade, i.e., arbitrage between low-priced and high-priced EU member states, can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any products, if approved in those countries.

U.S. Healthcare and Other Reform

In the U.S., federal and state governments continue to propose and pass legislation designed to reform delivery of, or payment for, health care, which include initiatives to reduce the cost of healthcare. For example, in March 2010, the U.S. Congress enacted the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act (the "Healthcare Reform Act"), which expanded health care coverage through Medicaid expansion, implemented the "individual mandate" for health insurance coverage (by imposing a tax penalty on individuals who did not obtain insurance) and changed the coverage and reimbursement of drug products under government healthcare programs.

Beyond the Healthcare Reform Act, there have been ongoing healthcare reform efforts, including efforts focused on drug pricing and payment. For example, federal legislation eliminated a statutory cap on Medicaid drug rebate program rebates effective January 1, 2024. As another example, the Inflation Reduction Act ("IRA") of 2022 includes a number of changes intended to address rising prescription drug prices in Medicare Parts B and D, with varying implementation dates. These changes include caps on Medicare Part D out-of-pocket costs, Medicare Part B and Part D drug price inflation rebates, a new Medicare Part D manufacturer discount drug program (replacing the ACA Medicare Part D coverage gap discount program) and a drug price negotiation program for

certain high spend Medicare Part B and D drugs (with the first list of drugs announced in 2023). Subsequent to the enactment of the IRA, in 2024, the Biden Administration announced its commitment to expanding certain IRA reforms. The focus on drug pricing and payment reform is likely to continue under the new Trump Administration. Other potential healthcare reform efforts under the Trump Administration could affect access to healthcare coverage or the funding of health care benefits. There is significant uncertainty regarding the nature or impact of any such reform implemented by the Trump Administration through executive action or by Congress.

Healthcare reform efforts have been and may continue to be subject to scrutiny and legal challenge. For example, with respect to the Healthcare Reform Act, tax reform legislation was enacted that eliminated the tax penalty established for individuals who do not maintain mandated health insurance coverage beginning in 2019 and, in 2021, the U.S. Supreme Court dismissed the latest judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the Healthcare Reform Act. As another example, revisions to regulations under the federal anti-kickback statute would remove protection for traditional Medicare Part D discounts offered by pharmaceutical manufacturers to pharmacy benefit managers and health plans. Pursuant to court order, the removal was delayed and recent legislation imposed a moratorium on implementation of the rule until January 2032. As another example, the IRA drug price negotiation program has been challenged in litigation filed by various pharmaceutical manufacturers and industry groups.

There have also been efforts by government officials or legislators to implement measures to regulate prices or payment for pharmaceutical products, including legislation on drug importation. Recently, there has been considerable public and government scrutiny of pharmaceutical pricing and proposals to address the perceived high cost of pharmaceuticals. There have also been recent state legislative efforts to address drug costs, which generally have focused on increasing transparency around drug costs or limiting drug prices. Certain state legislation has been subject to legal challenges.

General legislative cost control measures may also affect reimbursement for our products. The Budget Control Act of 2011, as amended, resulted in the imposition of reductions in Medicare (but not Medicaid) payments to providers in 2013 and remains in effect through 2032 unless additional Congressional action is taken. Any significant spending reductions affecting Medicare, Medicaid or other publicly funded or subsidized health programs that may be implemented and/or any significant taxes or fees that may be imposed on us could have an adverse impact on our results of operations.

Healthcare or budget reform at the federal or state level could affect demand for, or pricing of, our products or product candidates if approved for sale and may adversely affect our future business and financial results. We cannot, however, predict the ultimate content, timing or effect of any such reform, or its impact on our business operations.

Competition

The pharmaceutical and biotechnology industries are intensely competitive, and any product or product candidate developed by us competes or would likely compete with existing drugs and therapies. There are many pharmaceutical companies, biotechnology companies, public and private universities, government agencies and research organizations that compete with us in developing various approaches to the treatment of rare, neuromuscular and other diseases. Many of these organizations have substantially greater financial, technical, manufacturing and sales and marketing resources than we do. Several of them have developed or are developing therapies that could be used for treatment of the same diseases that we are targeting. In addition, some of these competitors have significantly greater commercial infrastructures than we have. Our ability to compete successfully depends largely on:

- the efficacy, safety and reliability of our products and product candidates;
- the dosing, strength, convenience and other product profile attributes of our products and product candidates;
- product acceptance by physicians and other health-care providers;
- protection of our proprietary rights and the level of generic or innovative competition;
- the ability to have freedom to operate to commercialize our products and product candidates;
- our ability to supply commercial quantities of a product meeting FDA specifications to the market and the cost of supplying our products and product candidates;
- our ability to complete clinical development and obtain regulatory approvals for our product candidates;
- obtaining reimbursement for product use in approved indications and the price of our products;
- our ability to recruit and retain skilled employees; and
- the availability of substantial capital resources to fund development and commercialization activities.

EXONDYS 51, VYONDYS 53 and AMONDYS 45 were the first three disease modifying therapeutics approved by the FDA for the treatment of Duchenne for patients with a confirmed mutation that is amenable to exon 51 skipping, exon 53 skipping or exon 45 skipping, respectively. ELEVIDYS was the first gene therapy approved for the treatment of patients aged 4 through 5 years with Duchenne with a confirmed mutation in the Duchenne gene. However, in the field of Duchenne alone, these products and those in our

pipeline face a variety of competitors who either have FDA approval or are being clinically developed for the treatment of Duchenne. For example, Nippon Shinyaku Co. Ltd. ("Nippon") announced on August 13, 2020 that the FDA approved VILTEPSO (viltolarsen) injection for patients with Duchenne who are amenable to exon 53 skipping therapy. On March 25, 2020, Nippon announced that the Japanese Ministry of Health, Labor, and Welfare approved Viltepso Intravenous Infusion 250 mg (viltolarsen) for the treatment of patients with Duchenne who are amendable to exon 53 skipping therapy making it the first non-steroidal treatment for Duchenne approved in Japan. Nippon has announced plans to pursue global registration for viltolarsen. Beyond Viltolarsen, Nippon is developing NS-089 and NS-050 for the treatment of patients living with Duchenne that have mutations amenable to exons 44 and 50, respectively.

In addition, there are many companies who have announced clinical development plans for the treatment of Duchenne, including the following:

- Wave Life Sciences ("Wave") announced in December 2023 that it initiated dosing in a Phase 2 potentially registrational, open-label clinical trial evaluating WVE-N531, its exon 53 skipping product candidate, and announced positive interim data in September 2024.
- Dyne Therapeutics is in clinical trials for Dyne-251 for the treatment of patients living with Duchenne that have mutations amenable to exon 51 skipping.
- Avidity Biosciences, Inc. is in clinical trials for AOC 1044 for the treatment of patients living with Duchenne that have mutations amenable to exon 44 skipping.
- PepGen is in clinical trials for PGN-ED051 for the treatment of patients living with Duchenne that have mutations amenable to exon 51.
- Entrada Therapeutics is in clinical trials for ENTR-601-44 for the treatment of patients living with Duchenne that have mutations amenable to exon 44.
- Regenxbio is in clinical trials for RGX-202 for the treatment of patients living with Duchenne.
- BioMarin Pharmaceutical announced a Phase I/II trial in certain European countries for its oligonucleotide candidate BMN-351 for the treatment of ambulatory patients with Duchenne that have mutations amenable to exon 51 skipping.
- Solid Biosciences announced a Phase I/II trial in the USA for its gene therapy candidate SGT-003.

There are several companies in addition to those mentioned above that are pursuing disease modifying programs for Duchenne that are at the pre-clinical stage or clinical stage. These companies are pursuing oligonucleotides, gene transfer therapy or gene editing. Other companies continue to pursue development and approval of products for the treatment of Duchenne and their products may or may not prove to be safer and/or more efficacious than the products and product candidates in our Duchenne pipeline. Regarding any of these competitors, it is unknown if clinical development of these or other compounds is planned or would be continued.

Additionally, companies have product candidates with mechanisms of action distinct from ours in different stages of development or approval in Duchenne which we believe could be seen as complementary to exon skipping and not a direct replacement of our products or product candidates at this time. We also believe that other biotechnology and pharmaceutical companies share a focus on RNA-targeted, gene therapy and gene editing drug discovery and development.

Several companies and institutions have also entered into collaborations or other agreements for the development of product candidates, including, but not limited to, gene therapy, gene editing, RNA, small molecule, cell therapy or antibody therapeutics that are potential competitors to therapies being developed by us in the muscular dystrophy, neuromuscular, CNS and rare disease space.

For additional information on the various risks posed by competition, refer to Part I, Item 1A. Risk Factors of this Annual Report on Form 10-K.

Human Capital Resources

As of December 31, 2024, we had 1,372 employees globally, 51% of whom hold advanced degrees. Of these employees, 68% are engaged directly in research and development activities and 32% are in selling and general and administration. Our voluntary employee turnover rate for 2024 was 4.6%. None of our employees in the U.S. are covered by collective bargaining agreements and we consider relations with our employees to be good.

We face intense competition for qualified and specialized employees from other pharmaceutical and biotechnology companies, universities and government entities, and we are committed to rewarding, supporting, and developing our employees who make it possible to deliver on our strategy. To that end, we offer a comprehensive total rewards package that includes

market-competitive pay, broad-based equity grants and bonuses, healthcare benefits, retirement savings plans, paid time off and family leave, caregiving support, fitness subsidies, and an Employee Assistance Program. We also offer robust learning opportunities for employees at every stage in their career and provide annual training to employees on various topics.

General Corporate Information

We were originally incorporated in the State of Oregon on July 22, 1980, and on June 6, 2013, we reincorporated in the State of Delaware. Our principal executive offices are located at 215 First Street, Suite 415, Cambridge, MA 02142 and our telephone number is (617) 274-4000. Our common stock is quoted on the Nasdaq Global Select Market under the symbol "SRPT".

While we achieve revenue from our products in the U.S. and through distribution of our products through our EAP outside the U.S., we may incur operating losses in the near term associated with our ongoing operations, research and development activities and potential business development activities. For more information about our revenues and operating losses, see *Item 7, Management's Discussion and Analysis of Financial Condition and Results of Operations*.

As of December 31, 2024, we had approximately $1,503.5 million of cash, cash equivalents, restricted cash and investments, consisting of $1,103.0 million of cash and cash equivalents, $384.9 million of investments and $15.6 million of restricted cash. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next 12 months. In addition to pursuing additional cash resources through public or private financings, we may also seek to enter into contracts, including collaborations or licensing agreements with respect to our technologies, with third parties, including government entities.

Where You Can Find Additional Information

We make available free of charge through our corporate website, www.sarepta.com, our annual reports, quarterly reports, current reports, proxy statements and all amendments to those reports as soon as reasonably practicable after such material is electronically filed or furnished with the SEC. These reports may also be obtained without charge by submitting a written request via mail to Investor Relations, Sarepta Therapeutics, Inc., 215 First Street, Suite 415, Cambridge, MA 02142 or by e-mail to investorrelations@sarepta.com. Our internet website and the information contained therein or incorporated therein are not intended to be incorporated into this Annual Report on Form 10-K. In addition, the SEC maintains an Internet site that contains reports, proxy and information statements, and other information regarding reports that we file or furnish electronically with the SEC at www.sec.gov.

We have adopted a Code of Business Conduct and Ethics and written charters for our Audit Committee, Compensation Committee and Nominating and Corporate Governance Committee. Each of the foregoing is available on our website at www.sarepta.com under "For Investors—Corporate Governance." In accordance with SEC rules, we intend to disclose any amendment (other than any technical, administrative, or other non-substantive amendment) to the above code, or any waiver of any provision thereof with respect to any of our executive officers, on our website within four business days following such amendment or waiver. In addition, we may use our website as a means of disclosing material non-public information and for complying with our disclosure obligations under Regulation Fair Disclosure promulgated by the SEC. These disclosures will be included on our website under the "For Investors" section.

Item 1A. Risk Factors.

Set forth below and elsewhere in this report and in other documents we file with the SEC are descriptions of risks and uncertainties that could cause actual results to differ materially from the results contemplated by the forward-looking statements contained in this report. Because of the following factors, as well as other variables affecting our operating results, past financial performance should not be considered a reliable indicator of future performance and investors should not use historical trends to anticipate results or trends in future periods. The risks and uncertainties described below are not the only ones facing us. Other events that we do not currently anticipate or that we currently deem immaterial also affect our results of operations and financial condition.

Risks Related to Our Business

We are highly dependent on the commercial success of our products. We may not be able to meet expectations with respect to sales of our products or maintain profitability and positive cash-flow from operations.

The commercial success of our products continues to depend on, and the commercial success of any future products would depend on, a number of factors attributable to one of our products or the products of our competitors, including, but not limited to:

- the effectiveness of our sales, managed markets, marketing efforts and support for our products;
- the generation and dissemination of new data analyses and the consistency of any new data with prior results, whether they support a favorable safety, efficacy and effectiveness profile of our products and any potential impact on our FDA accelerated approval status and/or FDA package insert for our products;
- the effectiveness of our ongoing commercialization activities, including negotiating and entering into any additional commercial, supply and distribution contracts, ongoing manufacturing efforts and hiring any additional personnel as needed to support commercial efforts;
- our ability to timely comply with FDA post-marketing requirements and commitments, including through successfully conducting additional studies that confirm clinical efficacy, effectiveness and safety of our products and acceptance of the same by the FDA and medical community since continued approval may be contingent upon verification of a clinical benefit in confirmatory trials, particularly in light of FDA's expanded expedited withdrawal procedures as set forth in FDORA;
- the occurrence of any side effects, adverse reactions or misuse, or any unfavorable publicity in these areas;
- the generation of evidence describing payers, patients and/or societal value of our products;
- whether we can consistently manufacture our products and product candidates at acceptable costs;
- the rate and consistency with which our products are prescribed by physicians, which depends on physicians' views on the safety, effectiveness and efficacy of our products;
- our ability to secure and maintain adequate reimbursement for our products, including the duration of the prior-authorization as well as the number and duration of re-authorization processes required for patients who initially obtained coverage by third parties, including by government payors, managed care organizations and private health insurers;
- our ability to obtain and maintain patent protection for our products, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing on the proprietary rights of third parties;
- the development, commercialization or pricing of competing products or therapies for the treatment of Duchenne, or its symptoms, and the existence of competing clinical trials;
- our ability to increase awareness of the importance of genetic testing and knowing/understanding Duchenne mutations, and identifying and addressing procedural barriers to obtaining therapy;
- our ability to remain compliant with evolving laws and regulations that apply to us and our commercial activities;
- the actual market-size, ability to identify patients and the demographics of patients eligible for our products, which may be different than expected;
- the sufficiency of our drug supply to meet commercial and clinical demands and standards, which are negatively impacted by various factors, including when our projections on the potential number of amenable patients and their average weight are inaccurate; the potential impacts of future pandemics; if regulatory requirements increase our drug supply needs; if our current drug supply is destroyed or negatively impacted at our manufacturing sites, storage sites or

in transit; failure to meet cGMP requirements; or if we encounter delays expanding the number of patients on our products and portions of our products' supply expire before sale;

- our ability to obtain regulatory approvals to commercialize our product candidates, and to commercialize our products in markets outside of the U.S.;
- the process leading to a patient's first infusion of our products and any future commercial products may be slower for certain patients. For example, the time to first infusion may take longer if a patient chooses to put in an intravenous port, which eases access to the vein. In addition, the capacity of any infusion centers responsible for the administration of ELEVIDYS may impact timing. Delays in the process prior to infusion could negatively impact the sales of our products, including any future gene therapy products; and
- the exercise by Roche of its option to obtain an exclusive license to commercialize one or more of our Duchenne products beyond ELEVIDYS outside of the U.S. and Roche's subsequent commercialization efforts.

We experience significant fluctuations in sales of our products from period to period and, ultimately, we may never generate sufficient revenues from our products to maintain profitability or sustain our anticipated levels of operations.

Even though EXONDYS 51, VYONDYS 53, AMONDYS 45 and ELEVIDYS have received accelerated approval from the FDA, they face future post-approval development and regulatory requirements, which present additional challenges for us to successfully navigate.

The accelerated approvals for EXONDYS 51, VYONDYS 53 and AMONDYS 45 granted by the FDA were based on an increase in the surrogate biomarker of dystrophin in skeletal muscles observed in some patients treated with these products. The accelerated approval for ELEVIDYS in non-ambulatory patients granted by the FDA was based on an effect on the surrogate endpoint of expression of ELEVIDYS micro-dystrophin, the protein produced by ELEVIDYS. These products are subject to ongoing FDA requirements governing labeling, packaging, storage, advertising, promotion and recordkeeping, and we are required to submit additional safety, efficacy and other post-marketing information to the FDA.

Under the accelerated approval pathway, continued approval may be contingent upon verification of a clinical benefit in confirmatory trials. These post-approval requirements and commitments may not be feasible and/or could impose significant burdens and costs on us; could negatively impact our development, manufacturing and supply of our products; and could negatively impact our financial results. Failure to meet post-approval commitments and requirements, including completion of enrollment and in particular, any failure to obtain safety and efficacy data that supports clinical benefits from our ongoing and planned studies of our products, could lead to negative regulatory action from the FDA and/or withdrawal of regulatory approval of EXONDYS 51, VYONDYS 53, AMONDYS 45 or ELEVIDYS. The recently enacted FDORA has expanded FDA's expedited withdrawal procedures for drugs approved via the accelerated approval pathway if a sponsor fails to conduct any required post-approval study with due diligence.

Manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with FDA requirements, including cGMP regulations. Drug product manufacturers are required to continuously monitor and report adverse events from clinical trials and commercial use of the product. If we or a regulatory agency discover previously unknown adverse events or events of unanticipated severity or frequency, a regulatory agency may establish additional regulatory requirement including, among other things, labeling changes, implementation of risk evaluation and mitigation strategy program, or additional post-marketing studies or clinical trials. If we or a regulatory agency discover previously unknown problems with a product, such as problems with a facility where the API or drug product is manufactured or tested, a regulatory agency may impose restrictions on that product and/or the manufacturer, including removal of specific product lots from the market, withdrawal of the product from the market, suspension of manufacturing or suspension of clinical trials using the same manufacturing materials. Sponsors of drugs approved under FDA accelerated approval provisions also are required to submit to the FDA, at least 30 days before initial use, all promotional materials intended for use after the first 120 days following marketing approval. If we or the manufacturing facilities for our products fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw or alter the conditions of our marketing approval;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- suspend any ongoing clinical trials;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;

- refuse to approve pending applications or supplements to applications submitted by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements;
- seize or detain products, refuse to permit the import or export of products or require us to initiate a product recall; or
- refuse to allow us to enter into supply contracts, including government contracts.

We are subject to uncertainty relating to reimbursement policies which, if not favorable, could hinder or prevent the commercial success of our products and/or product candidates.

Our ability to successfully maintain and/or increase sales of our products in the U.S. depends in part on the coverage and reimbursement levels set by governmental authorities, private health insurers and other third-party payors. Third party payors are increasingly challenging the effectiveness of, and the prices charged for medical products and services. We may not be able to obtain or maintain adequate third-party coverage or reimbursement for our products, and/or we may be required to provide discounts or rebates on our products in order to obtain or maintain adequate coverage.

We expect that private insurers will continue to consider the efficacy, effectiveness, cost-effectiveness and safety of our products, including any new data and analyses that we are able to collect and make available in a compliant manner, in determining whether to approve reimbursement for our products and at what levels. If there are considerable delays in the generation of new evidence or if any new data and information we collect is not favorable, third party insurers may make coverage decisions that negatively impact sales of our products. We continue to have discussions with payors, some of which may eventually deny coverage. We may not receive approval for reimbursement of our products from additional insurers on a satisfactory rate or basis, in which case our business would be materially adversely affected. In addition, obtaining these approvals can be a time consuming and expensive process. Our business would be materially adversely affected if we are not able to maintain favorable coverage decisions and/or fail to receive additional favorable coverage decisions from third party insurers, in particular during re-authorization processes for patients that have already initiated therapy. Our business could also be adversely affected if government health programs, private health insurers, including managed care organizations, or other reimbursement bodies or payors limit the indications for which our products will be reimbursed or fail to recognize approval or accelerated approval and surrogate endpoints as clinically meaningful.

Furthermore, we cannot predict to what extent an economic recession, changes in fiscal policy or general increase in unemployment rates may disrupt global healthcare systems and access to our products or result in a widespread loss of individual health insurance coverage due to unemployment or trends in employee attrition, a shift from commercial payor coverage to government payor coverage, or an increase in demand for patient assistance and/or free drug programs, any of which would adversely affect access to our products and our net sales.

In some foreign countries, particularly Canada and the countries of Europe, Latin America and Asia Pacific, the pricing and reimbursement of prescription pharmaceuticals is subject to strict governmental control. In these countries, pricing and reimbursement negotiations with governmental authorities can take 12 to 24 months or longer after the receipt of regulatory approval and product launch. In order to obtain favorable reimbursement for the indications sought or pricing approval in some countries, we may be required to collect additional data, including conducting additional studies. Furthermore, several countries around the world have implemented government measures to either freeze or reduce pricing of pharmaceutical products. If reimbursement for our products is unavailable in any country in which reimbursement is sought, limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed. In addition, many foreign countries reference to other countries' official public list price, hence an unsatisfactory price level in one country could consequently impinge negatively upon overall revenue.

We expect to experience pricing pressures in connection with the sale of our current and future products due to a number of factors, including current and future healthcare reforms and initiatives by government health programs and private insurers (including managed care plans) to reduce healthcare costs, the scrutiny of pharmaceutical pricing, the ongoing debates on reducing government spending and additional legislative proposals. These healthcare reform efforts or any future legislation or regulatory actions aimed at controlling and reducing healthcare costs, including through measures designed to limit reimbursement, restrict access or impose unfavorable pricing modifications on pharmaceutical products, could impact our and our partners' ability to obtain or maintain reimbursement for our products at satisfactory levels, or at all, which could materially harm our business and financial results.

Additionally, ELEVIDYS and our gene therapy product candidates represent novel approaches to treatment that will call for new levels of innovation in both pricing, reimbursement, payment and drug access strategies. Current reimbursement models may not accommodate the unique factors of our gene therapy product and product candidates, including high up-front costs, lack of long-term efficacy and safety data and fees associated with complex administration, dosing and patient monitoring requirements. Hence, it may be necessary to restructure approaches to payment, pricing strategies and traditional payment models to support these therapies.

The downward pressure on healthcare costs in general has become intense. As a result, increasingly high barriers are being erected to the entry of new products. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market

and sell our products and product candidates will be harmed. The manner and level at which reimbursement is provided for services related to our products and product candidates (e.g., for administration of our products to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and limit our ability to market or sell our products.

Healthcare policy reform and other governmental and private payor initiatives may have an adverse effect upon, and could prevent commercial success of our products and product candidates.

The U.S. government and individual states continue to aggressively pursue healthcare reform, which includes ongoing attempts to manage utilization as well as control and/or lower the cost of prescription drugs and biologics. See "*Item 1. Business – Government Regulation – U.S. Healthcare and Other Reform*" There is no assurance that federal or state health care reform will not adversely affect our future business and financial results, and we cannot predict how future federal or state legislative, judicial or administrative changes relating to healthcare policy will affect our business.

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid and private insurance healthcare costs, including proposed or implemented reforms involving price controls, waivers from Medicaid drug rebate law requirements, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs and implementing new requirements for, or eliminating caps on, rebates paid on products under government healthcare programs. We anticipate that the Trump Administration and Congress, state legislatures and the private sector will continue to consider and may adopt healthcare policies intended to curb rising healthcare costs, and specifically prescription drug costs. These cost containment measures may include, among other possible actions, implementation or modification of:

- controls on government funded reimbursement for drugs;
- mandatory discount requirements under certain government sponsored programs;
- caps on drug reimbursement under commercial insurance;
- challenges to the pricing of drugs or limits or prohibitions on reimbursement for specific products through other means;
- reform of drug importation laws;
- delegation of decision making to state Medicaid agencies and waiver of coverage and reimbursement requirements;
- mechanisms utilized by managed care organizations to control utilization of drugs and other health care;
- prohibition on direct-to-consumer advertising or drug marketing practices.

In recent years, there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their commercial products, which has resulted in several Congressional inquiries and proposed and enacted state and federal legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for pharmaceutical products.

Additionally, in its 2024 decision in *Loper Bright Enterprises v. Raimondo*, the U.S. Supreme Court overruled the "Chevron doctrine," which gives deference to regulatory agencies' statutory interpretations in litigation against federal government agencies, such as the FDA, the Centers for Medicare & Medicaid Services ("CMS") and other federal agencies where the law is ambiguous. The *Loper* decision could result in additional legal challenges to regulations and guidance issued by federal agencies, including the FDA and the CMS, on which we rely. Any such legal challenges, if successful, could have a material impact on our business. Additionally, the *Loper* decision may result in increased regulatory uncertainty, inconsistent judicial interpretations, and other impacts to the agency rulemaking process, any of which could adversely impact our business and operations. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action or as a result of legal challenges, either in the US or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, our business could be materially harmed.

We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third party coverage and reimbursement may be enacted in the future or what effect such legislation, regulations or policies would have on our business. Any cost containment measures, including those listed above, or other healthcare system reforms that are adopted, could significantly decrease the available coverage and the price we might establish for our products and product candidates, which would have an adverse effect on our net revenues and operating results.

Our products may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our potential profitability and future business prospects.

The commercial success of our products, particularly in the U.S., depends upon the level of market adoption by patients, payors and healthcare providers. If our products do not achieve an adequate level of market adoption for any reason, or if market adoption does not persist, our potential profitability and our future business prospects will be severely adversely impacted. The degree of market acceptance of our products depends on a number of factors, including:

- our ability to demonstrate to the medical and payor community, including specialists who may purchase or prescribe our products, the clinical efficacy, effectiveness and safety of our products as the prescription products of choice for their respective indications;
- the effectiveness of our sales and marketing organizations and distribution networks;
- the ability of patients or providers to be adequately reimbursed for our products in a timely manner from government and private payors;
- the ability to timely demonstrate to the satisfaction of payors real world effectiveness and the economic, humanistic, societal and clinical benefits of our products;
- the burden or efficiency of payer prior authorization processes and the ability of families and physicians to navigate them;
- the actual and perceived efficacy and safety profile of our products, particularly if unanticipated adverse events related to our products' treatment arise and create safety concerns among potential patients or prescribers or if new data and analyses we obtain for our products do not support, or are interpreted by some parties to not support, the efficacy of our products; and
- the efficacy and safety of our other exon-skipping and gene therapy product candidates and third parties' competitive therapies.

Further, the potential commercial success of our product candidates as well as ELEVIDYS will depend on additional factors, including the capacity of any infusion centers responsible for the administration of our product candidates and ELEVIDYS.

ELEVIDYS and our gene therapy product candidates may be perceived as insufficiently effective, unsafe or may result in unforeseen adverse events. Failure of other gene therapy programs, negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of ELEVIDYS or our gene therapy product candidates and harm our ability to conduct our business or obtain regulatory approvals for ELEVIDYS or our gene therapy product candidates.

Gene therapy remains a newly applied technology, with only a few gene therapy products approved to date in the U.S., the EU or elsewhere, including ELEVIDYS. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available.

In addition, ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed their intentions to further regulate biotechnology. More restrictive regulations or claims that our products or product candidates are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

More restrictive government regulations or negative public opinion would harm our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our gene therapy product candidates or demand ELEVIDYS or any other products we may develop. For example, earlier gene therapy trials led to several well-publicized adverse events, including death, and other gene therapy trials have failed to demonstrate efficacy. Lack of efficacy and/or serious adverse events related to clinical trials we, our strategic partners or other companies conduct, even if such adverse events are not ultimately attributable to the relevant product candidates or products, and/or failed commercialization of gene therapy products may result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our

product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

We may not be able to expand the global footprint of our products outside of the U.S.

In addition to receiving accelerated approval in the U.S., EXONDYS 51 has been approved for marketing in Israel, Libya, Kuwait, and Georgia, AMONDYS 45 in Libya and Kuwait, and VYONDYS 53 in Libya and Kuwait. We may not receive approval to commercialize these products in additional countries. Our partner for ELEVIDYS, Roche, has received certain approvals for ELEVIDYS in territories outside of the U.S. In November 2016, we submitted a MAA for eteplirsen to the EMA and the application was validated in December 2016. As we announced on June 1, 2018, the CHMP of the EMA adopted a negative opinion for eteplirsen. In September 2018, the CHMP of the EMA confirmed its negative opinion for eteplirsen, and the European Commission adopted the CHMP opinion in December 2018. During 2019, we sought follow-up EMA scientific advice for eteplirsen. Once data from our ongoing studies are available, we plan to evaluate future engagement with the EMA on potential next steps.

In order to market any product in a country outside of the U.S., we must comply with numerous and varying regulatory requirements for approval in those countries regarding demonstration of evidence of the product's safety and efficacy and governing, among other things, labeling, distribution, advertising, and promotion, as well as pricing and reimbursement of the product. Obtaining marketing approval in a country outside of the U.S. is an extensive, lengthy, expensive and uncertain process, and the regulatory authority may reject an application or delay, limit or deny approval of any of our products for many reasons, including:

- we may not be able to demonstrate to the satisfaction of regulatory authorities outside the U.S. the risk benefit of our products;
- the results of clinical trials may not meet the level of statistical or clinical significance required for approval by regulatory authorities outside the U.S.;
- regulatory authorities outside the U.S. may disagree with the adequacy (number, design, size, controls, conduct or implementation) of our clinical trials prior to granting approval, and we may not be able to generate the required data on a timely basis, or at all;
- regulatory authorities outside the U.S. may conclude that data we submit to them fail to demonstrate an appropriate level of safety or efficacy of our products, or that our products' respective clinical benefits outweigh their safety risks;
- regulatory authorities outside the U.S. may not accept data generated at our clinical trial sites or require us to generate additional data or information;
- regulatory authorities outside the U.S. may impose limitations or restrictions on the approved labeling of our products, thus limiting intended users or providing an additional hurdle for market acceptance of the product;
- regulatory authorities outside the U.S. may identify deficiencies in the manufacturing processes, or may require us to change our manufacturing process or specifications; and
- regulatory authorities outside the U.S. may adopt new or revised approval policies and regulations.

Approval procedures vary among countries and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ significantly from that required to obtain approval in the U.S. In particular, in many foreign countries, it is required that a product receives pricing and reimbursement approval before the product can be distributed commercially. Many foreign countries undertake cost-containment measures that could affect pricing or reimbursement of our products. This can result in substantial delays, and the price that is ultimately approved in some countries may be lower than the price for which we expect to offer our products.

Marketing approval in one country does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may have a negative effect on the approval process in others. Failure to obtain marketing approval in other countries or any delay or setback in obtaining such approval would impair our ability to develop foreign markets for our products and could adversely affect our business and financial condition. In addition, failure to obtain approval in one country or area may affect sales under the EAP in other countries or areas. Even if we are successful in obtaining regulatory approval of our products in additional countries, our revenue earning capacity will depend on commercial and medical infrastructure, pricing and reimbursement negotiations and decisions with third party payors, including government payors.

In addition, we have granted Roche an exclusive option to obtain an exclusive license to commercialize certain products, including eteplirsen, golodirsen and casimersen, outside of the U.S. If this option is exercised, Roche will have sole control over and decision-making authority with respect to the commercialization of such products outside the U.S.

Historical revenues from eteplirsen, golodirsen and casimersen through our EAP outside the U.S. may not continue and we may not be able to continue to distribute our products through our EAP.

We established a global EAP for our products in some countries where these products currently have not been approved. While we generate revenue from the distribution of these products through our EAP, we cannot predict whether historical revenues from this program will continue, whether we will be able to continue to distribute our products through our EAP, or whether revenues will exceed revenues historically generated from sales through our EAP. Reimbursement of aforementioned products through our EAPs may cease to be available if authorization for an EAP expires or is terminated. For example, healthcare providers in EAP jurisdictions may not be convinced that their patients benefit sufficiently from our products or alternatively, may prefer to wait until such time as our products are approved by a regulatory authority in their country before prescribing any of our products. Even if a healthcare provider is interested in obtaining access to our products for its patient through the EAP, the patient may not be able to obtain access to our products if funding for the drug is not secured. Also geo-political changes and challenges might negatively impinge upon future revenue generated through our EAP.

Our business and financial results have not yet been materially adversely affected by the ongoing conflict between Russia and Ukraine, or the conflict in the Middle-East. However, access to and reimbursement for patients in those regions through our EAP and consequently, our ability to generate revenue from sales of our products in Russia, Ukraine or the Middle East may be adversely affected in the future. The US and other nations have raised the possibility of sanctions on companies that do business with Russia or its allies, including Belarus. We also may be adversely impacted by sanctions imposed on third parties with which we do business, such as third-party distributors and service providers of our EAP.

Any failure to maintain revenues from sales of our products through our EAP and/or to generate revenues from commercial sales of these products exceeding historical sales due to geo-political challenges like those potentially resulting from the ongoing conflict between Russia and Ukraine or the instability in the Middle-East, could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

Failure to obtain or maintain regulatory exclusivity for our products could result in our inability to protect our products from competition and our business may be adversely impacted. If a competitor obtains an authorization to market the same or substantially same product before a product of ours is authorized in a given country and is granted regulatory exclusivity, then our product may not be authorized for sale as a result of the competitor's regulatory exclusivity and as a result, our investment in the development of that product may not be returned.

In addition to any patent protection, we rely on various forms of regulatory exclusivity to protect our products. During the development of our products, we anticipate any one form of regulatory exclusivities becoming available upon approval of our products. Implementation and enforcement of regulatory exclusivity, which may consist of regulatory data protection and market protection, varies widely from country to country. Failure to qualify for regulatory exclusivity, or failure to obtain or maintain the extent or duration of such protections that we expect in each of the markets for our products due to challenges, changes or interpretations in the law or otherwise, could affect our revenues for our products or our decision on whether to market our products in a particular country or countries or could otherwise have an adverse impact on our results of operations. We are not guaranteed to receive or maintain regulatory exclusivity for our current or future products, and if our products that are granted orphan status were to lose their status as orphan drugs or the data or marketing exclusivity provided for orphan drugs, our business and operations could be adversely affected.

Due to the nature of our products and product candidate pipeline, in addition to NCE exclusivity and new biologic exclusivity, orphan drug exclusivity is especially important for our products that are eligible for orphan drug designation. For eligible products, we plan to rely on orphan drug exclusivity to maintain a competitive position. If we do not have adequate patent protection for our products, then the relative importance of obtaining regulatory exclusivity is even greater. While orphan status for any of our products, if granted or maintained, would provide market exclusivity for the time periods specified above upon approval, we would not be able to exclude other companies from obtaining regulatory approval of products using the same or similar active ingredient for the same indication during or beyond the exclusivity period applicable to our product on the basis of orphan drug status (e.g., seven years in the U.S.). For example, the exclusivity period for EXONDYS 51 ended in September 2023. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process. A decision in 2021 by the U.S. Court of Appeals for the Eleventh Circuit in *Catalyst Pharmaceuticals, Inc. vs. Becerra* regarding interpretation of the Orphan Drug Act's exclusivity provisions as applied to drugs and biologics approved for orphan indications narrower than the product's orphan designation has the potential to significantly broaden the scope of orphan exclusivity for such products. While the FDA has since taken the position that it will continue to apply orphan drug exclusivity only on the basis of the specific indication, the Supreme Court's recent decision in 2024 in *Loper Bright Enterprises v. Raimondo* has the potential to impact how the Agency applies the *Catalyst* decision. Our ability to obtain or seek to work around orphan exclusivity, as well as our ability to retain orphan exclusivity that the FDA previously has recognized for our products, may be impacted depending

on how the *Catalyst* decision is ultimately implemented. Legislation has been introduced to amend the Orphan Drug Act in a way that may prevent these effects of the Catalyst decision, but it is unclear if or when such legislation could be enacted.

In addition, we may face risks with maintaining regulatory exclusivities for our products, and our protection may be circumvented, even if maintained. For instance, orphan drug exclusivity in the U.S. may be rescinded if (i) an alternative, competing product demonstrates clinical superiority to our product with orphan exclusivity; or (ii) we are unable to assure the availability of sufficient quantities of our orphan products to meet the needs of patients. Moreover, competitors may receive approval of different drugs or biologics for indications for which our prior approved orphan products have exclusivity. In Europe, the granted orphan exclusivity period may be reduced to six years if, at the end of the fifth year, it is established, in respect of the medicinal product concerned, that the criteria for orphan designation are no longer met, among other things, where it is shown on the basis of available evidence that the product is sufficiently profitable not to justify maintenance of market exclusivity. The granted market exclusivity may also be ineffective against a similar medicinal product where the originator is unable to supply sufficient quantities of the medicinal product or a competitor drug, although similar, is safer, more effective or otherwise clinically superior than the initial orphan drug. The scope of the orphan drug exclusivity in Europe may be modified after grant of the market authorization of the orphan product (*e.g.*, the approved therapeutic indication based on the benefit-risk assessment is narrower than or a subset of the designated orphan indication). Where the therapeutic indication being sought for approval does not fall within the scope of the designated orphan condition, a request should be sought for the designation decision to be amended. An amendment is possible only if the new condition differs slightly from that designated previously.

Thus, other companies may have received, or could receive, approval to market a product candidate that is granted orphan drug exclusivity for the same drug or similar drug and same orphan indication as any of our product candidates for which we plan to file an NDA, BLA or MAA. If that were to happen, our prior approved orphan products may face competition and any pending NDA, BLA or MAA for our product candidate for that indication may not be approved until the competing company's period of exclusivity has expired in the U.S. or the EU, as applicable. For example, in September 2021, the FDA issued guidance concerning its position on interpreting when gene therapy products would be considered the "same" or "different" for purposes of orphan drug exclusivity. The guidance states that if two gene therapy products have or use different vectors, the FDA generally intends to consider them to be "different" drugs. Further, according to the guidance, the FDA generally intends to consider vectors from the same viral group (e.g., AAV2 vs. AAV5) to be different, when the differences between the vectors impact factors such as tropism, immune response avoidance, or potential insertional mutagenesis. However, there is considerable uncertainty as to the interpretation of these guidelines. As illustrated by this guidance, orphan drug exclusivity as applied to gene therapy products is an evolving area subject to change and interpretation by the FDA and therefore, we cannot be certain as to how the FDA will apply those rules to ELEVIDYS or our gene therapy product candidates. Similarly, pursuant to the 2018 Commission Regulation, two gene therapy medicinal products are not considered similar when there are differences in the therapeutic sequence, viral vector, transfer system, regulatory sequences or manufacturing technology that significantly affect the biological characteristics and/or biological activity relevant for the intended therapeutic effect and/or safety attributes of the product.

If we are unable to successfully maintain and further develop internal commercialization capabilities, sales of our products may be negatively impacted.

We have hired and trained a commercial team and put in the organizational infrastructure we believe we need to support the commercial success of our products in the U.S. Factors that may inhibit our efforts to maintain and further develop commercial capabilities include:

- an inability to retain an adequate number of effective commercial personnel;
- an inability to train sales personnel, who may have limited experience with our company or our products, to deliver a consistent message regarding our products and be effective in educating physicians on how to prescribe our products;
- an inability to equip sales personnel with compliant and effective materials, including medical and sales literature to help them educate physicians and our healthcare providers regarding our products and their proper administration and educate payors on the safety, efficacy and effectiveness profile of our products to support favorable coverage decisions;
- unforeseen costs and expenses associated with maintaining and further developing an independent sales and marketing organization; and
- an inability to develop effective commercial, sales and marketing infrastructure to support new product launches.

If we are not successful in maintaining an effective commercial, sales and marketing infrastructure, we will encounter difficulty in achieving, maintaining or increasing projected sales of our products in the U.S., which would adversely affect our business and financial condition.

The patient population suffering from Duchenne, LGMDs, CMT 1A, FSHD and DM1 is small and has not been established with precision. If the actual number of patients is smaller than we estimate, our revenue and ability to achieve profitability may be adversely affected.

Duchenne, LGMD, and CMT 1A are rare, fatal genetic disorders. FSHD is a rare neuromuscular disease with an estimated U.S. prevalent population of approximately 13,000. DM1 is also a rare neuromuscular disease with an estimated U.S. prevalent population of approximately 30,000. Duchenne affects an estimated one in approximately every 3,500 to 5,000 males born worldwide, of which up to 13% are estimated to be amenable to exon 51 skipping, up to 8% are estimated to be amenable to exon 53 skipping and up to 8% are estimated to be amenable to exon 45 skipping. LGMDs as a class affect an estimated range of approximately one in every 14,500 to one in every 123,000 individuals. CMT is a group of peripheral nerve disorders affecting approximately one in every 2,500 individuals. CMT type 1A affects approximately 50,000 patients in the U.S. Our estimates of the size of these patient populations are based on a limited number of published studies as well as internal analyses. Various factors may decrease the market size of our products and product candidates, including the severity of the disease, patient demographics and the response of patients' immune systems to our products and product candidates. If the results of these studies or our analysis of them do not accurately reflect the relevant patient population, our assessment of the market may be inaccurate, making it difficult or impossible for us to meet our revenue goals, or to maintain profitability.

We face intense competition and rapid technological change, which may result in other companies discovering, developing or commercializing competitive products.

The biotechnology and pharmaceutical industries are highly competitive and subject to significant and rapid technological change, including the use of artificial intelligence ("AI"). We are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development in areas in which our products and product candidates are aimed. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with our products or product candidates. For example, we face competition in the field of Duchenne by third parties who are developing or who had once developed:

(i) exon skipping product candidates, such as Wave (targeting various exons, including 53 and 51), Nippon Shinyaku (targeting various exons, including 51 and 45, and notably for exon 53 for which it has received accelerated FDA approval for its product Viltepso (viltolarsen)), Dyne Therapeutics pursuing antibody-oligonucleotide conjugates for exons 44, 45, 51, and 53, Avidity Biosciences pursuing antibody-oligonucleotide conjugates for exons 44, 45 and 51, PepGen (notably for exon 51), SQY Therapeutics and BioMarin (BMN-351 for exon 51), Entrada;

(ii) gene therapies, such as Genethon and Solid (also in partnership with Ultragenyx), and Regenxbio;

(iii) gene editing, including CRISPR/Cas 9 approaches, such as Exonics Therapeutics (acquired by Vertex Pharmaceuticals), GenAssist, CRISPR Therapeutics, and Precision Biosciences;

(iv) other disease modifying approaches, such as PTC Therapeutics and Satellos, which has a small molecule candidate, ataluren, that targets nonsense mutations; and

(v) other approaches that may be palliative in nature or potentially complementary with our products and product candidates and that are or were once being developed including but not limited to, Santhera (Reveragen), Fibrogen, Capricor Therapeutics (in partnership with Nippon Shinyaku), BioPhytis, Italfarmaco (approved product Givinostat), Dystrogen and Edgewise Therapeutics. Although BioMarin announced on May 31, 2016 its intent to discontinue clinical and regulatory development of drisapersen as well as its other clinical stage candidates, BMN 044, BMN 045 and BMN 053, then-currently in Phase 2 studies for distinct forms of Duchenne, it further announced its intent to continue to explore the development of next generation oligonucleotides for the treatment of Duchenne. Indeed, BioMarin is conducting clinical trials for BMN-351, an oligonucleotide therapy. In addition, while Wave announced its intention to discontinue development of suvodirsen and suspend development of WVE-N531, it is conducting clinical trials for its exon 53 oligonucleotide, WVE-N531.

In addition, we are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development using platform technologies that may be viewed as competing with ours beyond and including those companies mentioned immediately above, such as Alnylam Pharmaceuticals, Inc., Arbutus (formerly Tekmira Pharmaceuticals Corp.), Deciphera Pharmaceuticals, Ionis Pharmaceuticals, Inc., Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Shire plc (now Takeda), Biogen, Moderna Therapeutics, Avidity, Dyne Therapeutics, Stoke Therapeutics, Ultragenyx, Sanofi and PepGen. Additionally, several companies and institutions have entered into collaborations or other agreements for the development of product candidates, including mRNA, gene therapy and gene editing (CRIPSR and AAV, among others) and small molecule therapies that are potential competitors for therapies being developed in the muscular dystrophy, neuromuscular and rare disease space, including, but not limited to, Astellas Pharma, Biogen Inc., Ionis, Alexion Pharmaceuticals, Inc., Sanofi, Shire (now Takeda), Eli Lilly, Alnylam Pharmaceuticals, Inc., Moderna Therapeutics, Inc., Akashi, Capricor Therapeutics (in partnership with Nippon Shinyaku), Oxford University, Exonics Therapeutics (acquired by Vertex Pharmaceuticals), and Editas Medicine.

If any of our competitors are successful in obtaining regulatory approval for any of their product candidates, it may limit our ability to enter into the market, gain market share or maintain market share in the Duchenne space or other diseases targeted by our platform technologies, products and product candidate pipeline.

It is possible that our competitors will succeed in developing technologies that, in addition to limiting the market size for our products or product candidates, impact the regulatory approval and post-marketing process for our products and product candidates, are more effective than our products or product candidates or would render our technologies obsolete or noncompetitive. Our competitors may, among other things, relative to our products or product candidates:

- develop safer or more effective products;
- implement more effective approaches to sales and marketing;
- develop less costly products;
- have lower cost of goods;
- receive more favorable reimbursement coverage;
- obtain preferred formulary status;
- obtain regulatory approval more quickly;
- have access to more manufacturing capacity;
- develop products that are more convenient and easier to administer;
- form more advantageous strategic alliances; or
- establish superior intellectual property positions.

Further, development and commercialization of ELEVIDYS and any expansion of its currently approved label, and development of our gene therapy product candidates, may compete with or supersede our current approved products, which may impact future revenues from sales of our current approved products. Our gene therapy product candidates are being developed for potential treatment of overlapping patient populations with our current approved products, and we have not determined if our gene therapy product candidates will be used in patients in combination with our existing approved products or in separate treatment regiments.

Our revenue could face competitive pressures for any of the above reasons. Moreover, if competing products are marketed in a territory in which we also have the authority to market our products, our sales may diminish, or our business could be otherwise materially adversely affected.

Future sales of ELEVIDYS may decrease sales growth, or reduce sales, of our PMO products, which could negatively impact our operating results, including through potential inventory write-offs.

Substantial overlap may exist between the addressable patient population for ELEVIDYS and the patient populations eligible for treatment with our PMO products. In the future, ELEVIDYS may be used in combination with our PMO products or may be adopted as a separate treatment regimen. Accordingly, ELEVIDYS may compete with our PMO products. As a result, successful commercialization of ELEVIDYS may reduce sales of our PMO products, potentially resulting in significant accounting charges relating to write-off of inventory if such inventory becomes in excess, obsolete or unusable.

We have entered into multiple collaborations and strategic transactions and may seek or engage in future strategic collaborations, alliances, acquisitions or licensing agreements or other relationships that complement or expand our business. We may not be able to complete such transactions, and such transactions, if executed, may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

In order to achieve our long-term business objectives, we actively evaluate various strategic opportunities on an ongoing basis, including licensing or acquiring products, technologies or businesses. We may face competition from other companies in pursuing such opportunities. This competition is most intense for approved drugs and late-stage drug candidates, which have the lowest risk in terms of probability of success but would have a higher risk and more immediate effect on our financial performance. Our ability to complete transactions may also be limited by applicable antitrust and trade regulation laws and regulations in the relevant U.S. and foreign jurisdictions in which we or the operations or assets we seek to acquire carry on business.

We have entered into multiple collaborations, including with Roche, Arrowhead, Nationwide, Duke University, Dyno Therapeutics, and Hansa Biopharma. We may not realize the anticipated benefits of such collaborations, and the anticipated benefits of any future collaborations or strategic relationships, each of which involves numerous risks, including:

- collaborators have significant discretion in determining the efforts and resources that they will apply to a collaboration;
- collaborators may not pursue development and commercialization of our products or product candidates based on clinical trial results, changes in their strategic focus due to the acquisition of competitive products, availability of funding, or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial, stop a clinical trial, abandon a product candidate, repeat or conduct new clinical trials, or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates, or otherwise undermine or devalue the efforts of our collaboration;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that cause the delay or termination of the research, development or commercialization of our products or product candidates, or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated and, if terminated, may eliminate our rights to commercialize certain product candidates or may result in a need for additional capital;
- failure to successfully develop the acquired or licensed drugs or technology or to achieve strategic objectives, including successfully developing and commercializing the drugs, drug candidates or technologies that we acquire or license;
- entry into markets in which we have no or limited direct prior experience or where competitors in such markets have stronger market positions;
- disruption of our ongoing business, distraction of our management and employees from other opportunities and challenges and retention of key employees;
- potential failure of the due diligence processes to identify significant problems, liabilities or other shortcomings or challenges of an acquired company, or acquired or licensed product or technology, including but not limited to, problems, liabilities or other shortcomings or challenges with respect to intellectual property, product quality, safety, accounting practices, employee, customer or third-party relations and other known and unknown liabilities;
- liability for activities of the acquired company or licensor before the acquisition or license, including intellectual property infringement claims, violations of laws, commercial disputes, tax liabilities, and other known and unknown liabilities;
- exposure to litigation or other claims in connection with, or inheritance of claims or litigation risk as a result of an acquisition or license, including but not limited to, claims from terminated employees, customers, former equity holders or other third-parties;
- difficulty in integrating the products, product candidates, technologies, business operations and personnel of an acquired asset or company; and
- difficulties in the integration of the acquired company's departments, systems, including accounting, human resource and other administrative systems, technologies, books and records, and procedures, as well as in maintaining uniform standards, controls, including internal control over financial reporting required by the Sarbanes-Oxley Act of 2002 and related procedures and policies.

For example, we will have limited influence and control over the development and commercialization activities of Roche in the territories in which it leads development and commercialization of ELEVIDYS, and if the exclusive option is exercised, in the territories in which it may lead commercialization of certain other products or product candidates. Roche's development and commercialization activities in the territories where it is the lead party may adversely impact our own efforts in the U.S. Failure by Roche to meet its obligations under the Roche Agreement, to apply sufficient efforts at developing and commercializing collaboration products, or to comply with applicable legal or regulatory requirements, may materially adversely affect our business and our results

of operations. In addition, to the extent we rely on Roche to commercialize any products for which we obtain regulatory approval, we will receive less revenues than if we commercialized these products ourselves.

Even if we achieve the long-term benefits associated with strategic transactions, our expenses and short-term costs may increase materially and adversely affect our liquidity and short-term net income (loss). Future licenses or acquisitions could result in potentially dilutive issuances of our equity securities, the incurrence of debt, the creation of contingent liabilities, impairment or expenses related to goodwill, and impairment or amortization expenses related to other intangible assets, which could harm our financial condition.

Risks Related to the Development of our Product Candidates

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on the speed at which we can recruit eligible patients to participate in testing our product candidates. We have experienced delays in some of our clinical trials, and we may experience similar delays in the future. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology, delays in our ability to expand the labels of any of our approved products or termination of the clinical trials altogether.

We, or our strategic partners, may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a study, to complete clinical trials within the expected timeframe. Patient enrollment can be impacted by factors including, but not limited to:

- design and complexity and/or commitment of participation required in the study protocol;
- size of the patient population;
- diagnostic capabilities within patient population;
- eligibility criteria for the study in question;
- clinical supply availability;
- delays in participating site identification, qualification and subsequent activation to enroll;
- perceived risks and benefits of the product candidate under study, including as a result of adverse effects observed in similar or competing therapies;
- proximity and availability of clinical trial sites for prospective patients;
- availability of competing therapies and clinical trials;
- competition of site efforts to facilitate timely enrollment in clinical trials;
- participating site motivation;
- patient referral practices of physicians;
- activities of patient advocacy groups;
- ability to monitor patients adequately during and after treatment; and
- severity of the disease under investigation.

In particular, each of the conditions for which we plan to evaluate our product candidates are rare genetic diseases with limited patient pools from which to draw for clinical trials. Further, because newborn screening for these diseases is not widely adopted, and it can be difficult to diagnose these diseases in the absence of a genetic screen, we may have difficulty finding patients who are eligible to participate in our studies. The eligibility criteria of our clinical trials will further limit the pool of available study participants. Additionally, the process of finding and diagnosing patients may prove costly. The treating physicians in our clinical trials may also use their medical discretion in advising patients enrolled in our clinical trials to withdraw from our studies to try alternative therapies. In addition, pandemics and other national or regional health emergencies may impact patient ability and willingness to travel to clinical trial sites as a result of quarantines and other restrictions, which may negatively impact enrollment in our clinical trials.

We may not be able to initiate or continue clinical trials if we cannot enroll the required eligible patients per protocol to participate in the clinical trials required by the FDA or the EMA or other regulatory agencies. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with contract research organizations ("CROs") and physicians;
- different standards for the conduct of clinical trials;
- our inability to locate qualified local consultants, physicians and partners;
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment;
- ability to procure and deliver necessary clinical trial materials needed to perform the study; and
- inability to implement adequate training at participating sites remotely when in person training cannot be completed.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business and on our ability to maintain our accelerated approval in the U.S.

Failures or delays in the commencement or completion of ongoing and planned clinical trials of our product candidates could negatively impact commercialization efforts; result in increased costs; and delay, prevent or limit our ability to gain regulatory approval of product candidates and to generate revenues and continue our business.

Successful completion of clinical trials at each applicable stage of development is a prerequisite to submitting a marketing application to the regulatory agencies and, consequently, the ultimate approval and commercial marketing of any of our product candidates for the indications in which we develop them. We do not know whether any of our clinical trials, or those with our strategic partners, will begin or be completed, and results announced, as planned or expected, if at all, as the commencement and completion of clinical trials and announcement of results is often delayed or prevented for a number of reasons, including, among others:

- denial by the regulatory agencies of permission to proceed with our planned clinical trials or any other clinical trials we may initiate, or placement of a clinical trial on hold;
- delays in filing or receiving approvals of additional INDs that may be required;
- negative and/or unanticipated results from our ongoing non-clinical trials or clinical trials;
- challenges in identifying, recruiting, enrolling and retaining patients to participate in clinical trials;
- challenges with subject compliance within clinical trials;
- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the CROs/ vendors involved in the clinical trial;
- negotiate contracts and other related documents with clinical trial parties and institutional review boards, such as informed consents, CRO agreements and site agreements, which can be subject to extensive negotiations that could cause significant delays in the clinical trial process, with terms possibly varying significantly among different trial sites and CROs and possibly subjecting us to various risks;
- inadequate quantity or quality of supplies of a product candidate or other materials necessary to conduct clinical trials, for example as a result of delays in defining and implementing the manufacturing process for materials used in pivotal trials or for the manufacture of larger quantities or other delays or issues arising in the manufacturing of sufficient supply of finished drug product;
- difficulties obtaining IRB approval, and equivalent (Ethics Committees or ECs) approval for sites outside the U.S., to conduct a clinical trial at a prospective site or sites;
- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;
- delays or problems in analyzing data, or the need for additional analysis or data or the need to enroll additional patients;

- the occurrence of serious adverse events or unexpected drug-related side effects experienced by patients in a clinical trial or unexpected results in ongoing non-clinical trials;
- delays in validating endpoints utilized in a clinical trial;
- delays in validating outcome assessments needed in a clinical trial;
- our inability to have formal meetings with the regulatory agencies or to interact with them on a regular basis;
- our inability to satisfy the requirements of the regulatory agencies to commence clinical trials, such as developing potency assays and lot release specifications that correlate with the activity or response of the product candidate or other CMC requirements;
- the regulatory agencies disagreeing with our clinical trial design and our interpretation of data from clinical trials, or changing the requirements for approval even after the regulatory authority has reviewed and commented on the design for our clinical trials;
- reports from non-clinical or clinical testing of competing therapies that raise safety or efficacy concerns; and
- the recruitment and retention of employees, consultants or contractors with the required level of expertise.

Any inability to complete successfully pre-clinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties, as well as our ability to maintain our accelerated approvals. In addition, manufacturing or formulation changes to our product candidates often require additional studies to demonstrate comparability of the modified product candidates to earlier versions. Clinical study delays also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which impairs our ability to successfully commercialize our product candidates and harms our business and results of operations.

Clinical development is lengthy and uncertain. Clinical trials of our product candidates may be delayed, and certain programs may never advance in the clinic or may be more costly to conduct than we anticipate, any of which could have a material adverse impact on our business.

Clinical testing is expensive and complex and can take many years to complete, and its outcome is inherently uncertain. We may not be able to initiate, may experience delays in, or may have to discontinue clinical trials for our product candidates as a result of numerous unforeseen events, including:

- the FDA, other regulators, IRBs, or ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site for any number of reasons, including concerns regarding safety and aspects of the clinical trial design;
- we may experience delays in reaching, or fail to reach, agreement on favorable terms with prospective trial sites and prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- the outcome of our pre-clinical studies and our early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results;
- we may be unable to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful;
- clinical trials of any product candidates may fail to show safety or efficacy, or produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional nonclinical studies or clinical trials, or we may decide to abandon product development programs;
- differences in trial design between early-stage clinical trials and later-stage clinical trials make it difficult to extrapolate the results of earlier clinical trials to later clinical trials;
- pre-clinical and clinical data are often susceptible to varying interpretations and analyses, and many product candidates believed to have performed satisfactorily in pre-clinical studies and clinical trials have nonetheless failed to obtain marketing approval; and
- regulators may elect to impose a clinical hold, or we or our investigators, IRBs, or ethics committees may elect to suspend or terminate clinical research or trials for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable benefit risk ratio. For example, in the past, we have received clinical holds from the FDA. Although these holds have generally not materially affected our

development timelines, there is no assurance that any future hold would not have a material adverse effect. A clinical hold, or any of the above factors, may be out of our control and could materially impair our development timelines, expenses and results of operations.

Results from pre-clinical and early-stage clinical trials may not be indicative of safety or efficacy in late-stage clinical trials, and pre-clinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, which could prevent or significantly delay their regulatory approval.

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive pre-clinical and clinical trials, that the product candidate is safe and effective in humans. Ongoing and future pre-clinical and clinical trials, including those with our strategic partners, of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. For example, although we believe the data for SRP-9003 collected to date are positive, the additional data we collect may not be consistent with the pre-clinical and/or early clinical data or show a safe benefit that warrants further development or pursuit of a regulatory approval.

Furthermore, success in pre-clinical and early clinical trials does not ensure that the subsequent trials will be successful, nor does it predict final results of a confirmatory trial. Some of our clinical trials were conducted with small patient populations and were not blinded or placebo-controlled, making it difficult to predict whether the favorable results that we observed in such trials will be repeated in larger and more advanced clinical trials. For example, recent announcements for SRP-9003 include: in March 2022, we announced 24-month functional data from two clinical trial participants in the high-dose cohort, and 36-month functional data from three clinical trial participants in the low-dose cohort for SRP-9003. These data are based on small patient samples, and, given the heterogeneity of LGMD patients and potential lot-to-lot variability, the data may not be predictive of future results. In addition, we cannot assure that the results of additional data or data from any future trial will yield results that are consistent with the data presented, that we will be able to demonstrate the safety and efficacy of these product candidates, that later trial results will support further development, or even if such later results are favorable, that we will be able to successfully complete the development of, obtain accelerated, conditional or standard regulatory approval for, or successfully commercialize any of such product candidates. Similarly, we cannot provide assurances that data from our ongoing and planned studies with respect to our commercially approved products and product candidates will be positive and consistent or that the interpretation by regulators, such as the FDA or EMA, of the data we collect for our products or product candidates will be consistent with our interpretations.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent regulatory approval of product candidates, limit the commercial potential or result in significant negative consequences following any potential marketing approval.

Our product candidates may cause undesirable side effects. In addition to side effects caused by our product candidates, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur in our trials, we may decide, or the FDA, the EMA or other regulatory authorities could order us, to halt, delay or amend pre-clinical development or clinical development of our product candidates or we may be unable to receive regulatory approval of our product candidates for any or all targeted indications. Even if we are able to demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates and may harm our business, financial condition and prospects significantly.

If there are significant delays in obtaining, or if we are unable to obtain or maintain required regulatory approvals, we will not be able to commercialize our product candidates in a timely manner or at all.

The research, testing, manufacturing, labeling, approval, commercialization, marketing, selling and distribution of drug products are subject to extensive regulation by applicable local, regional and national regulatory authorities and regulations may differ from jurisdiction to jurisdiction. In the U.S., approvals and oversight from federal (e.g., the FDA), state and other regulatory authorities are required for these activities. Sale and marketing of our product candidates in the U.S. or other countries is not permitted until we obtain the required approvals from the applicable regulatory authorities. Of the large number of drugs in development in the biopharmaceutical industry, only a small percentage result in the submission of a marketing application to the FDA or an MAA to the EMA (or NCA of an EU member state) and even fewer are approved for commercialization.

Our ability to obtain the government or regulatory approvals required to commercialize any of our product candidates in any jurisdiction, including in the U.S. or the EU, cannot be assured, may be significantly delayed or may never be achieved for various reasons including the following:

- Our non-clinical, clinical, chemistry, manufacturing and controls and other data and analyses from past, current and future studies for any of our product candidates may not be sufficient to meet regulatory requirements for marketing application approvals. The regulatory authorities could disagree with our interpretations and conclusions regarding data we provide in connection with NDA, BLA or MAA submissions for one or more of our product candidates, and may delay, reject or refuse to accept for review, or approve any submission we make or identify additional requirements for product approval to be submitted upon completion, if ever. In addition, in the U.S., an FDA advisory committee could determine that our data are insufficient to provide a positive recommendation for approval of any NDA or BLA we submit to the FDA. Even if we meet FDA requirements and an advisory committee votes to recommend approval of an NDA or BLA submission, the FDA could still disagree with the advisory committee's recommendation and deny approval of a product candidate based on their review.
- The regulatory approval process for product candidates targeting orphan diseases, such as Duchenne, that use new technologies and processes, such as antisense oligonucleotide therapies, gene therapy and other alternative approaches or endpoints for the determination of efficacy is uncertain due to, among other factors, evolving interpretations of a new therapeutic class, the broad discretion of regulatory authorities, lack of precedent, small safety databases, varying levels of applicable expertise of regulators or their advisory committees, scientific developments, changes in the competitor landscape, shifting political priorities and changes in applicable laws, rules or regulations and interpretations of the same. As a result of uncertainty in the approval process for products intended to treat serious rare diseases, we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned NDAs, BLAs and MAAs for our product candidates, in a timely manner, or at all. Examples of such requests or requirements could include, but are not limited to, conducting additional or redesigned trials and procedures (e.g., additional safety data, patient muscle biopsies, dystrophin analyses and the use of assays), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, in the U.S., an FDA advisory committee or regulators may disagree with our data analysis, interpretations and conclusions at any point in the approval process, which could negatively impact the approval of our NDA or BLA or result in a decision by the Company not to proceed with an NDA or BLA submission for a product candidate based on feedback from regulators.
- We may not have the resources required to meet regulatory requirements and successfully navigate what is generally a lengthy, expensive and extensive approval process for commercialization of drug product candidates.

Any failure on our part to respond to these requirements in a timely and satisfactory manner could significantly delay or negatively impact confirmatory study timelines and/or the development plans we have for PMO, gene therapy-based product candidates or other product candidates. Responding to requests from regulators and meeting requirements for clinical trials, submissions and approvals may require substantial personnel, financial or other resources, which, as a small biopharmaceutical company, we may not be able to obtain in a timely manner or at all. In addition, our ability to respond to requests from regulatory authorities that involve our agents, third party vendors and associates may be complicated by our own limitations and those of the parties we work with. It may be difficult or impossible for us to conform to regulatory guidance or successfully execute our product development plans in response to regulatory guidance, including guidance related to clinical trial design with respect to any NDA, BLA or MAA submissions.

Even if our product candidates demonstrate safety and efficacy in clinical studies, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Disruptions at regulatory agencies that are unrelated to our products and product candidates could delay the review and approval of our products, which could adversely affect our business. For example, changes in government, the ability to hire and retain key personnel and statutory and regulatory changes could result in delays. In addition, government funding of regulatory, government agencies, and programs on which our operations may rely is subject to the impacts of political events, which are inherently unpredictable and fluid. Further, additional delays may result if an FDA Advisory Committee or other regulatory advisory group or authority recommends non-approval or restrictions on approval.

In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical studies and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. Furthermore, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates. Finally, some of our product candidates may require diagnostic tests to ensure we appropriately select patients suitable for treatment. If we are unable to successfully develop diagnostic tests for these product candidates, experience significant delays in doing so, or are unable to obtain required regulatory clearances or approvals for any diagnostic tests, the commercialization of our product candidates may be delayed or prevented. Even if we receive the required regulatory clearance or approvals for certain diagnostic tests, the commercial success of any of our product candidates that require such tests will be dependent upon the continued availability of such tests.

We are investing significant resources in the development of novel gene therapy product candidates. Only a few gene therapy products have been approved in the U.S. and the EU. If we are unable to show the safety and efficacy of these product candidates, experience delays in doing so or are unable to successfully commercialize at least one of these drugs, our business would be materially harmed.

We are investing significant resources in the development of our gene therapy product candidates. We believe that a significant portion of the long-term value attributed to our company by investors is based on the commercial potential of these product candidates. There can be no assurance that any development problems we experience in the future related to our gene therapy programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. Development problems and delays in one program may delay the development of other programs. Early results from ongoing clinical trials may differ materially from final results from such clinical trials. The results from pre-clinical and early clinical studies do not always accurately predict results in later, large-scale clinical trials. We may also experience delays in developing a sustainable, reproducible and commercial-scale manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical trials or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Currently, only a few gene therapy products have been approved in the western world. Given the few precedents of approved gene therapy products, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our gene therapy product candidates in the U.S., the EU or other jurisdictions. Approvals by the EMA and the EC may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene therapy products have evolved and may continue to change in the future. Within the FDA, the Center for Biologics Evaluation and Research ("CBER") regulates gene therapy products. Within the CBER, the review of gene therapy and related products is consolidated in the Office of Cellular, Tissue and Gene Therapies, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. The CBER works closely with the National Institutes of Health (the "NIH"). The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols. For example, on January 28, 2020, the FDA issued final guidance documents that updated draft guidance documents that were originally released in July 2018 to reflect recent advances in the field, and to set forth the framework for the development, review and approval of gene therapies. These final guidance documents pertain to the development of gene therapies for the treatment of specific disease categories, including rare diseases, and to manufacturing and long-term follow up issues relevant to gene therapy, among other topics. The FDA also issued a new guidance document in September 2021 describing the FDA's approach for determining whether two gene therapy products were the same or different for the purpose of assessing orphan drug exclusivity, as well as a final guidance document in January 2024 on human gene therapy product incorporating human genome editing. The FDA also issued a draft guidance in December 2023 that provides recommendations for developing a potency assurance strategy for gene therapy products. In addition, the FDA can put an IND on hold if the information in an IND is not sufficient to assess the risks in pediatric patients.

These regulatory review agencies, committees and advisory groups and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval studies, limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable requirements and guidelines, failure of which may lead to delayed or discontinued development of our product candidates.

If the anticipated or actual timing of marketing approvals for our gene therapy product candidates, or the market acceptance of these product candidates, if approved, including treatment reimbursement levels agreed to by third-party payors, do not meet the expectations of investors or public market analysts, the market price of our common stock would likely decline.

Because we are developing product candidates for the treatment of certain diseases in which there is little clinical experience and we are using new endpoints or methodologies, there is increased risk that the FDA, the EMA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent full or accelerated regulatory approval.

During the FDA review process, we will need to identify success criteria and endpoints such that the FDA will be able to determine the clinical efficacy and safety profile of our product candidates. As we are developing novel treatments for diseases in which there is little clinical experience with new endpoints and methodologies, such as gene therapy, there is heightened risk that the FDA, the EMA or other regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance. Achieving appropriate statistical power may be challenging for some of the ultra-rare genetically defined diseases we are targeting in our programs, especially if the acceptance of descriptive data is not yet established. In addition, different methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters may yield different statistical results. Even if we believe the data collected from clinical trials of our product candidates are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities. Pre-clinical and clinical data can be interpreted in different ways. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent full or accelerated regulatory approval.

If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld or withdrawn.

Fast track product, breakthrough therapy, priority review, or Regenerative Medicine Advanced Therapy ("RMAT") designation by the FDA, or access to the Priority Medicine scheme ("PRIME") by the EMA, for our product candidates, if granted, may not lead to faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek fast track, breakthrough therapy designation, RMAT designation, PRIME scheme access or priority review designation for our product candidates if supported by the results of clinical trials. A fast track product designation is designed to facilitate the clinical development and expedite the review of drugs intended to treat a serious or life-threatening condition which demonstrate the potential to address an unmet medical need. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. A RMAT designation is designed to accelerate approval for regenerative advanced therapies such as our gene therapy product candidates. Priority review designation is intended to speed the FDA marketing application review timeframe for drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. PRIME is a scheme built on the existing regulatory framework and tools already available such as scientific advice and accelerated assessment administered by the EMA to enhance support for the development of medicines that are considered of major public health interest, in particular from the viewpoint of therapeutic innovation to address an unmet medical need. By engaging with medicine developers early on, PRIME aims at improving scientific evidence-generation so that the data generated are suitable for evaluating a marketing-authorization application. Once admitted to the PRIME scheme, the sponsor will benefit from scientific and regulatory advice on the overall development plan and at major milestones, with an opportunity to involve stakeholders such as health technology bodies responsible for determining adoption of new treatment methods in the EU national health systems. PRIME-designated medicinal products may be eligible for accelerated assessment where the centralized assessment timeframe for 210 days, not counting procedural clock stops, can be reduced to 150 days.

For drugs and biologics that have been designated as fast track products or breakthrough therapies, or granted access to the PRIME scheme, interaction and communication between the regulatory agency and the sponsor of the trial can help to identify the most efficient path for clinical development. Sponsors of drugs with fast track products or breakthrough therapies may also be able to submit marketing applications on a rolling basis, meaning that the FDA may review portions of a marketing application before the sponsor submits the complete application to the FDA, if the sponsor pays the user fee upon submission of the first portion of the marketing application. For products that receive a priority review designation, the FDA's marketing application review goal is shortened to six months, as opposed to ten months under standard review. This review goal is based on the date the FDA accepts the marketing application for review. This application validation period typically adds approximately two months to the timeline for review and decision from the date of submission. RMAT designations will accelerate approval and will include all the benefits of fast

track and breakthrough therapy designations, including early interactions with the FDA, but the exact mechanisms have not yet been announced by the FDA.

Designation as a fast track product, breakthrough therapy, RMAT, PRIME, or priority review product is within the discretion of the regulatory agency. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a fast track product, breakthrough therapy, RMAT, PRIME, or priority review product, the FDA or the EMA may disagree and instead determine not to make such designation. In any event, the receipt of such a designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional regulatory procedures and does not assure ultimate marketing approval by the relevant agency. In addition, regarding fast track products and breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification as either a fast track product, RMAT, or a breakthrough therapy or, for priority review products, decide that period for FDA review or approval will not be shortened.

Even though our products are PRIME designated, the EMA may not accept that our products are eligible for expedited assessment. The EMA may decide to return to the standard assessment timeframe of 210 days if an application initially granted accelerated assessment does not meet the criteria for accelerated assessment.

We may not be able to advance all of our programs, and we may use our financial and human resources to pursue particular programs and fail to capitalize on programs that may be more profitable or for which there is a greater likelihood of success.

Our pipeline includes programs in various stages of development for a broad range of diseases and disorders. We plan to expand our pipeline through internal research and development and through strategic transactions. Because we have limited resources, we may not be able to advance all of our programs. We may also forego or delay pursuit of opportunities with certain programs or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

Risks Related to Third Parties

If we are unable to maintain our agreements with third parties to distribute our products to patients, our results of operations and business could be adversely affected.

We rely on third parties to commercially distribute our products to patients in the U.S. We have contracted with a third-party logistics company to warehouse our products and with distributors and specialty pharmacies to sell and distribute our products to patients. A specialty pharmacy is a pharmacy that specializes in the dispensing of medications for complex or chronic conditions that require a high level of patient education and ongoing management.

This distribution network requires significant coordination with our sales and marketing and finance organizations. In addition, failure to coordinate financial systems could negatively impact our ability to accurately report product revenue from our products. If we are unable to effectively manage the distribution process, the sales of our products, as well as any future products we may commercialize, could be delayed or severely compromised and our results of operations may be harmed.

In addition, the use of third parties involves certain risks, including, but not limited to, risks that these organizations will:

- not provide us with accurate or timely information regarding their inventories, the number of patients who are using our products or serious adverse events and/or product complaints regarding our products;
- not effectively sell or support our products;
- reduce or discontinue their efforts to sell or support our products;
- not devote the resources necessary to sell our products in the volumes and within the time frame we expect;
- be unable to satisfy financial obligations to us or others; or
- cease operations.

Any such events may result in decreased product sales, lower product revenue, loss of revenue, and/or reputational damage, which would harm our results of operations and business.

With respect to the pre-commercial distribution of our products to patients outside of the U.S., we have contracted with third party distributors and service providers to distribute our products in certain countries through our EAP. We will need to continue building out our network for commercial distribution in jurisdictions in which our products are approved, which will also require third party contracts. The use of distributors and service providers involves certain risks, including, but not limited to, risks that these organizations will not comply with applicable laws and regulations, or not provide us with accurate or timely information regarding serious adverse events and/or product complaints regarding our products. Any such events may result in regulatory actions that may include suspension or termination of the distribution and sale of our products in a certain country, loss of revenue, and/or reputational damage, which could harm our results of operations and business.

We rely on third parties to conduct some aspects of our early-stage research and pre-clinical and clinical development. The inadequate performance by or loss of any of these third parties could affect the development and commercialization of our product candidate development.

We have relied upon, and plan to continue to rely upon, third parties to conduct some aspects of our early-stage research and pre-clinical and clinical development with respect to certain of our product candidates, including our follow-on exon-skipping product candidates, gene therapy and gene editing product candidates. Our third-party collaborators may not commit sufficient resources or adequately develop our programs for these candidates. If our third-party collaborators fail to commit sufficient resources to any of our product candidates or to carry out their contractual duties or obligations, our programs related to any particular product candidate could be delayed, terminated, or unsuccessful. Furthermore, if we fail to make required payments to these third-party collaborators, including up-front, milestone, reimbursement or royalty payments, or to observe other obligations in our agreements with them, these third parties may not be required to perform their obligations under our respective agreements with them and may have the right to terminate such agreements. In addition, if our strategic partners experience regulatory delays for the development of their clinical product candidates, including clinical holds, our opportunities to commercialize products may be delayed.

We also have relied upon and plan to continue to rely upon third-party CROs to monitor and manage data completeness for our ongoing pre-clinical and clinical programs. We rely on these parties for execution of our pre-clinical and clinical trials, and we control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on collaborators and CROs does not relieve us of our regulatory responsibilities.

The individuals at our third-party collaborators and CROs who conduct work on our behalf, including their sub-contractors, are not always our employees, and although we participate in the planning of our early stage research and pre-clinical and clinical programs, we cannot control whether or not they devote sufficient time and resources or exercise appropriate oversight of these programs, except for remedies available to us under our agreements with such third parties. If our collaborators and CROs do not successfully carry out their contractual duties or obligations or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our pre-clinical and clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Our reliance on third parties requires us to share our proprietary information, which increases the possibility that a competitor will discover them or that our proprietary information will be misappropriated or inadvertently disclosed.

Our reliance on third-party collaborators requires us to disclose our proprietary information to these parties, which could increase the risk that a competitor will discover this information or that this information will be misappropriated or disclosed without our intent to do so. If any of these events were to occur, then our ability to obtain patent protection or other intellectual property rights could be irrevocably jeopardized, and costly, distracting litigation could ensue. Furthermore, if these third parties cease to continue operations and we are not able to quickly find a replacement provider or we lose information or items associated with our products or product candidates, our development programs may be delayed. Although we carefully manage our relationships with our third-party collaborators and CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

Some of the third parties we rely for early-stage research and pre-clinical development are located in China. There has been increased governmental focus in the U.S. on the role of Chinese companies in the life sciences industry. This focus has included U.S. legislative proposals, such as the proposed BIOSECURE Act, which is pending before the U.S. Senate. If enacted, the BIOSECURE Act would, among other things, prohibit U.S. federal agencies from entering into or renewing any contract with any entity that uses

biotechnology equipment or services produced or provided by a "biotechnology company of concern" to perform that contract with the government. If adopted, the BIOSECURE Act could cause us to seek to exit some or all of our arrangements with China-based service providers determined to be "biotechnology companies of concern" and transition these services to alternative companies.

Risks Related to Manufacturing

We currently rely on third parties to manufacture our products and to produce our product candidates; our dependence on these parties, including failure on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet commercial, EAP, clinical and pre-clinical product demand may impair the availability of product for commercial supply or to successfully support various programs, including research and development and the potential commercialization of additional product candidates in our pipeline.

We rely on, and expect to continue relying on for the foreseeable future, a limited number of third parties to manufacture and supply materials (including raw materials and subunits), API and drug product and to provide labeling and packaging of vials and storage of our products and product candidates. The limited number of third parties with facilities and capabilities suited for the manufacturing process of our products and product candidates creates a risk that we may not be able to obtain materials and APIs in the quantity and purity that we require. As of the date of this Annual Report, we have dual sourcing for the APIs and drug product for all three of our PMO commercial products and one source for ELEVIDYS drug substance and drug product manufacturing.

In addition, the process for adding new manufacturing capacity is lengthy and often causes delays in development efforts. Any interruption of the development or operation of those facilities due to, among other reasons, events such as a future pandemic, order delays for equipment or materials, equipment malfunctions, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters, such as earthquakes or fires, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in supply of our products, product candidates or materials. Any delay or interruption in the supply of finished products could hinder our ability to distribute our products to meet commercial demand or execute our commercialization plans on the timing that we expect, which could result in the loss of potential revenues, adversely affect our ability to gain market acceptance, or otherwise adversely affect our business, financial condition and prospects.

If these third parties cease providing quality manufacturing and related services to us, and we are not able to engage appropriate replacements in a timely manner, our ability to manufacture our products or product candidates in sufficient quality and quantity required for our planned commercial, pre-clinical and clinical or EAPs, our various product research, development and commercialization efforts would be adversely affected.

Furthermore, any problems in our manufacturing process or the facilities with which we contract make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs.

We, through our third-party manufacturers, seek to produce or produce supply of our products and product candidates. In light of the limited number of third parties with the expertise to produce our products and product candidates, the lead time needed to manufacture them, and the availability of underlying materials, we may not be able to, in a timely manner or at all, establish or maintain sufficient commercial and other manufacturing arrangements on the commercially reasonable terms necessary to provide adequate supply of our products and product candidates. Furthermore, we may not be able to obtain the significant financial capital that may be required in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for our products and product candidates, such parties may not comply with the terms and timelines they have agreed to for various reasons, some of which may be out of their or our control, which impacts our ability to execute our business plans on expected or required timelines in connection with the commercialization of our products and the continued development of our product candidates. When we enter into long-term manufacturing agreements that contain exclusivity provisions and /or substantial termination penalties, we constrain our operational flexibility.

We also rely on a third party to design, manufacture, obtain and maintain regulatory approval for necessary diagnostic tests for ELEVIDYS. Any delay or failure by us or our collaborators to develop or obtain regulatory approval of the necessary diagnostic tests could harm our business, possibly materially.

The operations at one of our partner sites could also be disturbed by man-made or natural disasters, public health pandemics or epidemics or other business interrupts such as potential supply chain disruptions caused by the ongoing conflict between Russia and Ukraine. In addition, the need to prioritize rated orders issued by the Federal Emergency Management Agency pursuant to the U.S. Defense Production Act could impact the manufacturing, supply chain and distribution of our products and product candidates.

Products intended for use in gene therapies are novel, complex and difficult to manufacture. We could experience production problems or inaccurately forecast demand, which could result in delays in commercialization or development of other gene therapy programs, limit the supply of our product candidates or future approved products or otherwise harm our business.

We currently have development, manufacturing and testing agreements with third parties to manufacture supplies of ELEVIDYS and our gene therapy product candidates. Several factors could cause production interruptions, including talent acquisition/retention, equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of suppliers.

The physical and chemical properties of biologics such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we employ multiple steps to control our manufacturing process to assure that the process works and the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in delay in product release, product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical and/or commercial-grade materials that meet FDA, EMA or other applicable foreign standards or specifications with consistent and acceptable production yields and costs. Lot failures or product recalls could cause us to delay clinical trials or product launches, or may result in an inability to fulfill demand for commercial supply of ELEVIDYS, or other future gene therapy products, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

In addition, the FDA, the EMA and other foreign regulatory authorities may require us to submit samples of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA or other foreign regulatory authorities may require that we not distribute a lot until the competent authority authorizes its release.

As our product candidates advance to later stage clinical trials, it is customary that various CMC aspects of the development program, such as manufacturing, formulation and other processes, and route of administration, may be altered to optimize the candidates and processes for scale-up necessary for later stage clinical trials and potential approval and commercialization. These changes may not produce the intended optimization, including production of drug substance and drug product of a quality and in a quantity sufficient for Phase 3 clinical stage development or for commercialization, which may cause delays in the initiation or completion of clinical trials and greater costs. We may also need to conduct additional studies to demonstrate comparability between newly manufactured drug substance and/or drug product for commercialization relative to previously manufactured drug substance and/or drug product for clinical trials. Demonstrating comparability may require us to incur additional costs or delay initiation or completion of clinical trials and, if unsuccessful, could require us to complete additional pre-clinical studies or clinical trials.

We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to operate our manufacturing process which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

In addition, if our third-party manufacturers are unable to satisfy requirements related to the manufacturing ELEVIDYS, our ability to meet commercial demand may be adversely impacted, which could result in the loss of potential revenues, adversely affect our ability to gain market acceptance of ELEVIDYS, or otherwise adversely affect our business, financial condition and prospects. ELEVIDYS is our first gene therapy product. We may not be able to accurately estimate commercial demand for this new type of product. If commercial demand for ELEVIDYS is greater than we estimate, we and our manufacturers may be unable to fulfill all orders for ELEVIDYS in a timely manner, which may adversely affect our business, financial condition and prospects.

Currently the capacity to produce our viral vectors or gene therapy product candidates at commercial levels is limited and the availability of sufficient GMP compliance capacity may result in delays in our development plans or increased capital expenditures, and the development and sales of any gene therapy products, if approved, may be materially harmed.

The third parties we use in the manufacturing process for our products and product candidates may fail to comply with cGMP regulations.

Our contract manufacturers are required to produce our materials, APIs and drug products under cGMP. We and our contract manufacturers are subject to periodic inspections by the FDA, the EMA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations. In addition, before we can begin to commercially manufacture our product candidates in third-party or our own facilities, we must obtain regulatory approval from the FDA, which includes a review of the manufacturing process and facility. A manufacturing authorization also must be obtained from the appropriate EU regulatory authorities and may be required by other foreign regulatory authorities. The timeframe required to obtain such approval or authorization is uncertain. In order to obtain approval, we need to demonstrate that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. In complying with cGMP, we are

obligated to expend time, money and effort in production, record keeping and quality control to seek to assure that the product meets applicable specifications and other requirements.

We do not have direct operational control over a third-party manufacturer's compliance with regulations and requirements. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of our products and product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively. Failure to achieve and maintain compliance with cGMP and other applicable government regulations, including failure to detect or control anticipated or unanticipated manufacturing errors, results in product recalls, clinical holds, delayed or withheld approvals, patient injury or death.

Failure by our contract manufacturers to adhere to applicable cGMP and other applicable government regulations, or our contract manufacturers experiencing manufacturing problems, may result in significant negative consequences, including product seizures or recalls, postponement or cancellation of clinical trials, loss or delay of product approval, fines and sanctions, loss of revenue, termination of the development of a product candidate, reputational damage, shipment delays, inventory shortages, inventory write-offs and other product-related charges and increased manufacturing costs. If we experience any of these consequences, the success of our commercialization of our products and/or our development efforts for our product candidates could be significantly delayed, fail or otherwise be negatively impacted.

We may not be able to successfully optimize manufacturing of our product candidates in sufficient quality and quantity or within targeted timelines, or be able to secure ownership of intellectual property rights developed in this process, which could negatively impact the commercial success of our products and/or the development of our product candidates.

Our focus remains on optimizing manufacturing, including for our product candidates, gene therapy and other programs. We may not be able to successfully increase manufacturing capacity for the production of materials, APIs and drug products, whether in collaboration with third party manufacturers or on our own, in a manner that is safe, compliant with cGMP conditions or other applicable legal or regulatory requirements, in a cost-effective manner, in a time frame required to meet our timeline for commercialization, clinical trials and other business plans, or at all.

Challenges complying with cGMP requirements and other quality issues arise during efforts to increase manufacturing capacity and scale up production. We experience such issues in connection with manufacturing, packaging and storage of our products and product candidates, and during shipping and storage of the APIs or finished drug product. In addition, in order to release our products for commercial use and demonstrate stability of product candidates for use in clinical trials (and any subsequent drug products for commercial use), our manufacturing processes and analytical methods must be validated in accordance with regulatory guidelines. Failure to successfully validate, or maintain validation of, our manufacturing processes and analytical methods or demonstrate adequate purity, stability or comparability of our products or product candidates in a timely or cost-effective manner, or at all, may undermine our commercial efforts. Failure to successfully validate our manufacturing processes and analytical methods or to demonstrate adequate purity, stability or comparability, will negatively impact the commercial availability of our products and the continued development and/or regulatory approval of our product candidates, which could significantly harm our business.

During our work with our third-party manufacturers to increase and optimize manufacturing capacity, they may make proprietary improvements in the manufacturing processes for our products or product candidates. We may not own or be able to secure ownership of such improvements or may have to share the intellectual property rights to those improvements. Additionally, we may need additional processes, technologies and validation studies, which could be costly and which we may not be able to develop or acquire from third parties. Failure to secure the intellectual property rights required for the manufacturing process needed for large-scale clinical trials or the continued development of our product candidates could cause significant delays in our business plans or otherwise negatively impact the continued development of our product candidates.

Risks Related to our Intellectual Property

Our success, competitive position and future revenue depend in part on our ability and the abilities of our licensors and other collaborators to obtain, maintain and defend the patent protection for our products, product candidates, and platform technologies, to preserve our trade secrets, and to prevent third parties from infringing on our proprietary rights.

We currently directly hold various issued patents and patent applications, or have exclusive license or option rights to issued patents and patent applications, in each case in the U.S. as well as other countries that protect our products, product candidates and platform technologies. We anticipate filing additional patent applications both in the U.S. and in other countries. Our success will depend, in significant part, on our ability to obtain, maintain and defend our U.S. and foreign patents covering our products, product candidates and platform technologies as well as preserving our trade secrets for these assets. The patent process is subject to numerous risks and uncertainties, and we can provide no assurance that we will be successful in obtaining, maintaining, or defending our patents.

Even when our patent claims are allowed, the claims may not issue, or in the event of issuance, may not be sufficient to protect our products, product candidates or platform technologies or may be challenged in post-grant proceedings by third parties.

The patent positions of pharmaceutical, biotechnology and other life sciences companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. This uncertainty is heightened for our PMO-based products and product candidates and gene therapy-based products and product candidates for which there has not been a significant number of patent litigations involving such technologies. Congress periodically considers changes to patent law, and that such changes could have adverse effects. No consistent policy regarding the breadth of claims allowed in biotechnology patents has emerged to date in the U.S. and tests used for determining the patentability of patent claims in all technologies are in flux. The USPTO and patent offices in other jurisdictions have often required that patent applications directed to pharmaceutical and/or biotechnology-related inventions be limited or narrowed substantially to cover only the specific innovations exemplified in the patent application, thereby limiting the scope of protection against competitive challenges. Accordingly, even if we or our licensors are able to obtain patents, the patents might be substantially narrower than anticipated. Thus, there is no assurance as to the degree and range of protections any of our patents, if issued, may afford us or whether patents will be issued. Patents which may be issued to us may be subjected to further governmental review that may ultimately result in the reduction of their scope of protection, and pending patent applications may have their requested breadth of protection significantly limited before being issued, if issued at all. The pharmaceutical, biotechnology and other life sciences patent situation outside the U.S. can be even more uncertain.

As a matter of public policy, there might be significant pressure on governmental bodies to limit the scope of patent protection or impose compulsory licenses for disease treatments that prove successful, particularly as a tactic to impose a price control. Additionally, competitors may leverage such pressure to enhance their ability to exploit these laws to create, develop and market competing products.

We may be able to assert that certain activities engaged in by our competitors infringe on our current or future patent rights. To the extent that we enforce our patents, an alleged infringer may deny infringement and/or counter-claim that our patents are not valid or enforceable, and if successful, could negatively impact our patent estate. We may not be able to successfully defend patents necessary to prevent competitors from developing, manufacturing, or commercializing competing product candidates or products. To the extent we assert infringement of a patent that covers a competing product candidate or product as well as our own product candidate(s) or product(s), or such a patent is otherwise challenged without our initiation, the patent protection for our own product candidate(s) or product(s) could be materially adversely affected should an infringing competitor be successful in challenging the validity, enforceability, or scope of our patent(s). Our patent rights might be challenged, invalidated, circumvented or otherwise not provide any competitive advantage. Defending our patent positions may require significant financial resources and could negatively impact other Company objectives. Even if we successfully enforce our patent rights against a competitor, we may not be able to recover adequate damages or obtain other desired relief.

Under the Hatch-Waxman Act, one or more motivated third parties may file an ANDA, seeking approval of a generic copy of an innovator product approved under the NDA pathway such as our PMO Products, or an NDA under Section 505(b)(2), for a new or improved version of the original innovator products. In certain circumstances, motivated third parties may file such an ANDA or NDA under Section 505(b)(2) as early as the so-called "NCE-1" date that is one year before the expiry of the five-year period of NCE exclusivity or more generally four years after NDA approval. The third parties are allowed to rely on the safety and efficacy data of the innovator's product, may not need to conduct clinical trials and can market a competing version of a product after the expiration or loss of patent exclusivity or the expiration or loss of regulatory exclusivity and often charge significantly lower prices. Upon the expiration or loss of patent protection or the expiration or loss of regulatory exclusivity for a product, the major portion of revenues for that product may be dramatically reduced in a very short period of time. If we are not successful in defending our patents and regulatory exclusivities, we will not derive the expected benefit from them. As such, a third party could be positioned to market an ANDA or Section 505(b)(2) product that competes with one of our products prior to the expiry of our patents if the third party successfully challenges the validity, enforceability, or scope of our patents protecting the product.

The patent landscape is continually evolving, and we may be able to assert that certain activities engaged in by third parties infringe our current or future patent rights. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights. As such, the patents and patent applications that we own, license, have optioned, and rely on for exclusivity for our product candidates may be challenged.

Uncertainty over intellectual property in the pharmaceutical and biotechnology industry has been the source of litigation and other disputes, which is inherently costly and unpredictable.

Litigation, interferences, oppositions, inter partes reviews, administrative challenges or other similar types of proceedings are, have been and may in the future be necessary in some instances to determine the validity and scope of certain of our proprietary rights, and in other instances to determine the validity, enforceability, scope or non-infringement of certain patent rights claimed by

third parties to be pertinent to the manufacture, use or sale of our product candidates or products. We may also face challenges to our patent and regulatory exclusivities covering our products by third parties, including manufacturers of generics and/or biosimilars who may choose to launch or attempt to launch their products before the expiration of our patents or regulatory exclusivity. Litigation, interferences, oppositions, inter partes reviews, administrative challenges or other similar types of proceedings are unpredictable and may be protracted, expensive and distracting to management. The outcomes of such proceedings could adversely affect the validity, enforceability, and scope of our patents or other proprietary rights, hinder our ability to manufacture and market our products, require us to seek a license for the infringed products or technology or result in the assessment of significant monetary damages against us that may exceed amounts, if any, accrued in our financial statements. An adverse determination in a judicial or administrative proceeding or a failure to obtain necessary licenses could prevent us from developing, manufacturing or selling our products. Furthermore, payments under any licenses that we are able to obtain would reduce our profits derived from our products. Any of these circumstances could result in financial, business or reputational harm to us or could cause a decline or volatility in our stock price.

Our business prospects will be impaired if third parties successfully assert that our products, product candidates, or platform technologies infringe proprietary rights of such third parties.

Similar to us, competitors continually seek intellectual property protection for their technology. Several of our development programs, particularly gene therapy programs, focus on therapeutic areas that have been the subject of extensive research and development by third parties for many years and have been protected with third party patent rights. Due to the amount of intellectual property in our various fields of technology, we cannot be certain that we do not infringe intellectual property rights of competitors or other third parties or that we will not infringe intellectual property rights of competitors or other third parties granted or created in the future. Moreover, activities we conduct or those conducted on our behalf in connection with the development of our product candidates may not be protected from infringement under the so-called Safe Harbor provision of 35 U.S.C. § 271(e)(1) and thus may be found to infringe the patent rights of third parties. Our competitors or other third parties might have obtained, or could obtain in the future, patents that threaten, limit, interfere with or eliminate our ability to make, use and sell our products, product candidates or platform technologies in important commercial markets.

Due to the nature of our various partnerships, collaborators, licensors, CROs, CMOs and the like, we may be subjected to claims of infringement arising from activities conducted by these third parties in connection with our product candidates, whether or not such activities are authorized by us. In addition, we may have contractual obligations to indemnify these partners from claims of infringement or declaratory relief. As a result, we may be subject to substantial unforeseen costs, distraction, and financial liability if a third party making such a claim was successful in obtaining a final judgment of infringement and validity.

In order to maintain or obtain freedom to operate for our products and product candidates, we may incur significant expenses, including those associated with entering into agreements with third parties that require milestone and royalty payments. Additionally, if we were to challenge the patent rights of our competitors or otherwise defend against allegations of infringement, misappropriation, breach of contract or related claims, we could incur substantial costs and ultimately might not be successful.

If our products, product candidates, or platform technologies are alleged to infringe or are determined to infringe enforceable proprietary rights of others, we could incur substantial costs and may have to:

- obtain rights or licenses from others, which might not be available on commercially reasonable terms or at all;
- abandon development of an infringing product candidate, or cease commercialization of an infringing product;
- redesign our products, product candidates or processes to avoid infringement;
- pay damages; and/or
- defend litigation or administrative proceedings which might be costly whether we win or lose, and which could result in a substantial diversion of financial and management resources.

Any of these events could result in product and product candidate development delays or cessation, and as such substantially harm our potential earnings, financial condition and operations. The patent landscape of our product candidates and products is continually evolving and multiple parties, including both commercial entities and academic institutions, may have rights to claims or may be pursuing additional claims that could provide these parties a basis to assert that our products, product candidates or platform technologies infringe on the intellectual property rights of such parties. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights.

Risks Related to our Business Operations

Failure to comply with healthcare and other regulations is subject to substantial penalties and our business, operations and financial condition could be adversely affected.

As a manufacturer of pharmaceuticals, within the U.S., certain federal and state healthcare laws and regulations apply to or affect our business. These laws may constrain the business or financial arrangements and relationships through which we conduct business, including how we conduct research regarding, market, sell, and distribute our products. The laws and regulations include:

- federal healthcare anti-kickback law, which prohibit, among other things, persons from soliciting, receiving or providing remuneration, directly or indirectly, to induce either the referral of an individual, for an item or service or the purchasing or ordering of a good or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid;
- federal false claims laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, information or claims for payment from Medicare, Medicaid or other third-party payors that are false or fraudulent;
- the Federal Food, Drug and Cosmetic Act, which among other things, strictly regulates drug product and medical device marketing, prohibits manufacturers from marketing such products for off-label use and regulates the distribution of samples;
- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers;
- federal laws that require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under government healthcare programs;
- the so-called “federal sunshine” law, which requires pharmaceutical and medical device companies to monitor and report certain financial interactions with teaching hospitals, physicians and certain non-physician practitioners as well as physician ownership interests to the federal government for re-disclosure to the public; and
- state law equivalents of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third party payor, including commercial insurers, state laws regulating interactions between pharmaceutical manufactures and healthcare providers, and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by federal laws, thus complicating compliance efforts.

The number and complexity of both federal and state laws continues to increase, and additional governmental resources are being used to enforce these laws and to prosecute companies and individuals who are believed to be violating them. We anticipate that government scrutiny of pharmaceutical sales and marketing practices and other activities will continue for the foreseeable future and subject us to the risk of government investigations and enforcement actions. Given the breadth of the laws and regulations, limited guidance for certain laws and regulations, and evolving government interpretations of the laws and regulations, governmental authorities may possibly conclude that our business practices are non-compliant.

We have implemented a compliance program, which is based on industry best practices and is designed to ensure that our activities comply with all applicable laws, regulations and industry standards. While our compliance program is intended to detect and prevent potential non-compliance, we cannot be certain that compliance will be assured. If our operations are found to be in violation of any of the laws described above or any other laws, rules or regulations that apply to us, we will be subject to penalties, including civil and criminal penalties, damages, fines and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could adversely affect our ability to operate our business and our financial results. Responding to government investigations, defending any claims raised, and any resulting fines, restitution, damages and penalties, settlement payments or administrative actions, as well as any related actions brought by stockholders or other third parties, could have a material impact on our reputation, business and financial condition and divert the attention of our management from operating our business. Even if we successfully defend against an action against us for violation of a law, the action and our defense could nonetheless cause us to incur significant legal expenses and divert our management’s attention from the operation of our business. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security, fraud and reporting laws may prove costly.

Failure to comply with data privacy and security laws and regulations could adversely affect our operating results and business.

We may collect, use, transfer, or otherwise process proprietary, confidential, and sensitive information, including personal information and health-related data, which subjects us to numerous evolving and complex data privacy and security obligations, including various laws, regulations, guidance, and industry standards. Within the U.S., there are numerous federal and state laws and regulations related to the privacy and security of personal information. For example, at the federal level, HIPAA, as amended, and its implementing regulations establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information. While we have determined that we are neither a "covered entity" nor a "business associate" directly subject to HIPAA, many of the U.S. health care providers with which we interact are subject to HIPAA, and we may have assumed obligations related to protecting the privacy of personal information. States are increasingly regulating the privacy and security of personal information. In some states, such as California and Washington, state privacy laws are even more protective than HIPAA. For example, the CCPA, regulates companies' use and disclosure of the personal information of California residents and grants California residents several rights with respect to their personal information. The CCPA also provides for civil penalties for violations, including statutory fines for noncompliance, as well as a limited private right of action in connection with certain data breaches, and establishes a new regulatory agency to implement and enforce the law. In addition, almost 20 other states have now passed comprehensive privacy laws that have taken effect or will come into effect at various times over the next few years. All of these evolving compliance and operational requirements impose significant costs that are likely to increase over time, may require us to modify our data processing practices and policies, divert resources from other initiatives and projects and could restrict the way services involving data are offered, all of which may adversely affect our results of operations. Certain state laws may be more stringent or broader in scope, or offer greater individual rights, than federal or other state laws, and such laws may differ from each other, which may complicate compliance efforts. State laws are changing rapidly and there is ongoing discussion in Congress of a new federal data protection and privacy law to which we may be subject. We will continue to monitor and assess the impact of these state laws, which may impose substantial penalties for violations, impose significant costs for investigations and compliance, and carry significant potential liability for our business.

Outside of the U.S., data protection laws, including the GDPR, which also forms part of the law of England and Wales, Scotland and Northern Ireland by virtue of section 3 of the European Union (Withdrawal) Act 2018 and as amended by the UK GDPR, also apply to some of our operations. The GDPR and UK GDPR increase our obligations with respect to clinical trials conducted in the member states of the EEA and the UK by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR and the UK GDPR increase the scrutiny that clinical trial sites located in the EEA and the UK should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the U.S. The GDPR and the UK GDPR impose substantial fines for breaches of data protection requirements, which can be up to four percent of global revenue or 20 million Euros (£17.5 million in the UK), whichever is greater, and they also confer a private right of action on data subjects for breaches of data protection requirements. Compliance with these directives is a rigorous and time-intensive process that requires review and updates that may increase our cost of doing business, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation and reputational harm in connection with our European and UK activities. Other governmental authorities around the world are considering and, in some cases, have enacted, similar privacy and data security laws. Failure to comply with federal, state and international data protection laws and regulations could result in government investigations and/or enforcement actions (which could include substantial civil and/or criminal penalties), private litigation and adverse publicity and could negatively affect our business, financial condition and results of operations.

Government pricing requirements, such as those under the Medicaid Drug Rebate Program, other federal government programs, and state price transparency laws, and their related reporting and payment obligations require strict adherence; our failure to adhere to such requirements could subject us to penalties, sanctions, and fines that could have a material adverse effect on our business, financial condition, results of operations, and growth prospects.

We participate in the Medicaid Drug Rebate Program, the Public Health Services ("PHS") 340B drug pricing program, the U.S. Department of Veterans Affairs, Federal Supply Schedule pricing program, and the Tricare Retail Pharmacy program, and have obligations to report the average sales price for certain drug products to the Medicare program. Compliance is challenging. Pricing and rebate calculations vary across products and programs, are complex, and are often subject to interpretation by us, governmental or regulatory agencies, and the courts, which can change and evolve over time.

Requirements are subject to challenge and change. For instance, the PHS 340B drug pricing program continues to be subject to legal and regulatory activity, including litigation, at the federal and state levels, and any related developments could alert the scope of the program and our obligation to offer discounts. Continued expansion of the PHS 340B drug pricing program and growth of entities claiming entitlement to 340B pricing, including in ways that may be inconsistent with the statutory scheme, could impact our revenue. Changes to the calculation of rebates under the Medicaid program could increase our Medicaid rebate obligations and decrease the prices charged to 340B covered entities. On September 20, 2024, CMS issued a final rule specifying penalties for misclassification of drugs, and otherwise altering manufacturer obligations, under the Medicaid Drug Rebate Program.

If we become aware that our reporting for a prior quarter or other time period was incorrect or has changed as a result of recalculation of pricing data, we generally are obligated to resubmit the corrected data and provide refunds or other reconciliations. Price recalculations may affect the ceiling price at which we are required to offer our products to certain customers under the PHS 340B drug pricing program and increase our general costs.

Civil monetary penalties can be applied if we are found to have knowingly submitted any false price or product information to the government, if we are found to have made a misrepresentation in the reporting of our average sales price, if we fail to submit the required price data on a timely basis, or if we are found to have charged certain customers more than the statutorily mandated ceiling price. The CMS also could decide to terminate our Medicaid Drug Rebate agreement. Our failure to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program and other governmental programs could negatively impact our financial results.

Several states have passed or are considering legislation that requires or purports to require companies to report pricing information, including proprietary pricing information. Such reporting requirements are not always clearly defined and failure to appropriately disclose in accordance with these requirements may lead to the imposition of penalties.

If we, our collaborators, or any third-party manufacturers engaged by us or our collaborators fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We, our collaborators, and any third-party manufacturers we engage are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of bio-hazardous materials. Our operations involve the use of hazardous materials, including organic and inorganic solvents and reagents. Although we believe that our activities conform in all material respects with such environmental laws, there can be no assurance that violations of these laws will not occur in the future as a result of human error, accident, equipment failure or other causes. Liability under environmental, health and safety laws can be joint and several and without regard to fault or negligence. The failure to comply with past, present or future laws could result in the imposition of substantial fines and penalties, remediation costs, property damage and personal injury claims, loss of permits or a cessation of operations, and any of these events could harm our business and financial condition. We expect that our operations will be affected by other new environmental, health and workplace safety laws on an ongoing basis, and although we cannot predict the ultimate impact of any such new laws, they may impose greater compliance costs or result in increased risks or penalties, which could harm our business.

Further, with respect to the operations of any current or future collaborators or third party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our product or product candidates, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product or product candidates.

Comprehensive tax reform in the U.S. and future guidance could adversely affect our business and financial condition.

The Tax Cuts and Jobs Act (the "TCJA") was enacted on December 22, 2017 in the U.S. The TCJA contains significant changes to corporate taxation, including reduction of the U.S. corporate tax rate from 35% to 21%, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, limitation of the tax deduction for interest expense, immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. On March 27, 2020, President Trump signed into law the "Coronavirus Aid, Relief, and Economic Security Act" or the CARES Act, which included certain changes in tax law intended to stimulate the U.S. economy in light of the COVID-19 outbreak, including temporary beneficial changes to the treatment of net operating losses, interest deductibility limitations and payroll tax matters.

We continue to monitor changes in tax laws in the U.S. and the impact of proposed and enacted legislation in the international jurisdictions in which the company operates, which could materially impact our tax provision, cash tax liability and effective tax rate.

Our ability to use net operating loss carryforwards and other tax attributes to offset future taxable income may be limited by provisions of the Internal Revenue Code, and it is possible that certain transactions or a combination of certain transactions may result in material additional limitations on our ability to use our net operating losses.

We have generated net operating loss and tax credit carryforwards in certain historical periods as we pursued our business strategy. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset a portion of future taxable income, if any, subject to expiration of such carryforwards in the case of carryforwards generated prior to January 1, 2018. In general, under Section 382 of the Internal Revenue Code, a corporation that undergoes an "ownership change" is subject to limitations on its ability to utilize its pre-change net operating losses and certain other tax assets (including R&D tax credits) to offset future taxable income. In general, an ownership change occurs if the aggregate stock ownership of certain stockholders increases by more than 50 percentage points over such stockholders' lowest percentage ownership during the testing period, which is generally three years. An ownership change could limit our ability to utilize our net operating loss and tax credit carryforwards for taxable years including or following such "ownership change." Such limitations may result in expiration of a portion of the net operating loss carryforwards incurred prior to 2018 before utilization and may be substantial. If such change has occurred or does occur, the tax benefits related to the net operating loss carryforwards and certain other tax assets may be limited or lost. Moreover, proposed U.S. Treasury Regulations promulgated under Section 382 of the Code could, if finalized, significantly impact a corporation's ability to use its pre-change net operating loss carryforwards or other attributes following an ownership change. Limitations imposed on the ability to use net operating losses and tax credits to offset future taxable income could require us to pay U.S. federal income taxes earlier than we estimated or than would have otherwise been required if such limitations were not in effect and could cause such net operating losses and tax credits to expire unused, in each case reducing or eliminating the benefit of such net operating losses and tax credits and potentially adversely affecting our financial position. Similar rules and limitations may apply for state income tax purposes. At the state level, there may also be periods during which the use of net operating loss carryforwards or other attributes is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. These net operating losses have been fully offset by a valuation allowance due to uncertainties surrounding our ability to realize these tax benefits.

The Inflation Reduction Act of 2022, among other things, implements a corporate book minimum tax ("BMT") 15% rate that could apply to consolidated groups of companies with adjusted financial statement income in excess of $1.0 billion over a three-year period. The BMT has various limitations, including a more restrictive limit on availability of net operating loss carryforwards, which if applied to us, could impact its cash tax liability and ability to utilize tax attributes.

In addition, many of the jurisdictions in which we operate have or are expected to adopt changes to tax laws as a result of the Base Erosion and Profit Shifting final proposals from the Organization for Economic Co-operation and Development and specific country anti-avoidance initiatives. In addition, the current proposal of the BMT may result in increases in tax imposed by non-U.S. jurisdictions. Such tax law changes and anti-avoidance initiatives increase uncertainty and may adversely affect our tax provision, cash tax liability and effective tax rate. The impact to the Company was not material in 2024 and the Company does not expect the impact to be material in future periods but will continue to monitor and evaluate new legislation and guidance.

Our employees, principal investigators, consultants and strategic partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and strategic partners. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the U.S. and abroad, report financial information or data accurately or disclose unauthorized activities to us. We adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Failure to retain our key personnel or an inability to attract and retain additional qualified personnel would cause our future growth and our ability to compete to suffer.

We are highly dependent on the efforts and abilities of the principal members of our senior management. Additionally, we have scientific personnel with significant and unique expertise in RNA-targeted therapeutics and gene therapy technologies. The loss of the services of any one of the principal members of our managerial team or staff may prevent us from achieving our business objectives.

The competition for qualified personnel in the biotechnology field is intense, and our future success depends upon our ability to attract, retain, motivate and support such personnel. In order to develop and commercialize our products successfully, we will be required to retain key management and scientific employees. In certain instances, we may also need to expand or replace our workforce and our management ranks. In addition, we rely on certain consultants and advisors, including scientific and clinical advisors, to assist us in the formulation and advancement of our research and development programs. Our consultants and advisors may be employed by other entities or have commitments under consulting or advisory contracts with third parties that limit their availability to us, or both. If we are unable to attract, assimilate or retain such key personnel, our ability to advance our programs would be adversely affected.

Turnover rates of key employees have varied substantially in recent years. Over the last few years, we have had several executive management changes. Leadership transitions can be inherently difficult to manage and may cause uncertainty or a disruption to our business or may increase the likelihood of turnover in other key officers and employees. If we lose the services of one or more of our senior management or key employees, or if one or more of them decides to join a competitor or otherwise to compete with us, our business could be harmed.

Risks Related to our Financial Condition and Capital Requirements

We have previously incurred operating losses and we may not maintain profitability.

While we generated an operating income of $218.1 million for the year ended December 31, 2024, our accumulated deficit to date was $4.2 billion. Although we currently have four commercially approved products in the U.S., we believe that it will take us some time to attain positive cash flow from operations. Since our products and product candidates target small patient populations, the per-patient drug pricing must be high in order to recover our development and manufacturing costs, fund adequate patient support programs, fund additional research and achieve profitability. We may be unable to maintain or obtain sufficient sales volumes at a price high enough to justify our product development efforts and our sales, marketing and manufacturing expenses.

We have generally incurred expenses related to research and development of our technologies and product candidates and from general and administrative expenses that we have incurred while building our business infrastructure. We anticipate that our expenses will increase substantially if and/or as we:

- continue the commercialization of our products in the U.S.;
- expand the global footprint of our products outside of the U.S.;
- establish our sales, marketing and distribution capabilities;
- continue our research, pre-clinical and clinical development of our product candidates;
- respond to and satisfy requests and requirements from regulatory authorities in connection with development and potential approval of our product candidates;
- initiate additional clinical trials for our product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- acquire or in-license other product candidates;
- maintain, expand and protect our intellectual property portfolio;
- increase manufacturing capabilities, including capital expenditures related to our real estate facilities and entering into manufacturing agreements;
- hire additional clinical, quality control and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

Because of the numerous risks and uncertainties associated with developing biopharmaceutical products, we are unable to predict our ability to continue to generate profitability or the extent of it.

Our existing and any future indebtedness could adversely affect our ability to operate our business.

On February 13, 2025, we entered into a $600.0 million revolving credit agreement with JPMorgan Chase Bank, N.A., as administrative agent and as collateral agent, the lenders party thereto, and Sarepta Therapeutics Investments, Inc., a Delaware corporation and wholly owned subsidiary, which we refer to as the "Credit Agreement". To the extent we draw amounts under the Credit Agreement in the future, our payment obligations under the Credit Agreement may reduce cash available to fund working capital, capital expenditures, research and development and general corporate needs. In addition, indebtedness incurred under the Credit Agreement bears interest at a variable rate, which would make us vulnerable to increases in interest rates. If interest rates increase, we would be required to pay additional interest on any indebtedness incurred under the Credit Agreement, which would further reduce cash available for our other business needs. We may not have sufficient funds, and may be unable to arrange for additional financing, to pay the amounts due under or refinance any indebtedness outstanding under the Credit Agreement, which is repayable on the maturity date, February 13, 2030.

Our obligations under the Credit Agreement are secured by substantially all of our assets and the assets of certain wholly owned material subsidiaries, subject to certain customary exceptions and exclusions. The security interest granted over our assets could limit our ability to obtain additional debt financing. In addition, the Credit Agreement contains financial covenants that are tested on the last day of each of the Company's fiscal quarters. These financial covenants include a (x) maximum secured net leverage ratio of 3.5:1.0, subject to a 4.0:1.0 covenant holiday following certain permitted acquisitions or permitted collaborations, and (y) minimum consolidated interest coverage ratio of 2.5:1.0. Failure to comply with the covenants in the Credit Agreement, including the financial covenants, could result in the acceleration of our obligations under the Credit Agreement. If an event of default (other than certain events of bankruptcy or insolvency) occurs and is continuing, JPMorgan Chase Bank, N.A. may terminate the commitments under the Credit Agreement and declare all or any portion of the outstanding principal amount of the loans plus accrued and unpaid interest to be due and payable. Upon the occurrence of certain events of bankruptcy or insolvency, all of the outstanding principal amount of the loans plus accrued and unpaid interest will automatically become due and payable. If such acceleration were to occur, it would materially and adversely affect our business, financial condition, operating results, cash flows and prospects.

Any outstanding indebtedness, combined with our other financial obligations, could increase our vulnerability to adverse changes in general economic, industry and market conditions, limit our flexibility in planning for, or reacting to, changes in our business and the industry and impose a competitive disadvantage compared to our competitors that have less debt, fewer operational restrictions or better debt servicing options.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights.

We may seek additional capital through a combination of private and public equity offerings, debt financings, collaborations and strategic and licensing arrangements. To the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, the ownership interest of our stockholders in our company may be diluted. In addition, the terms of any such securities may include liquidation or other preferences that materially adversely affect the rights of our stockholders. Debt financing, if available, may increase our fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaboration, strategic partnerships and licensing arrangements with third parties, we may have to relinquish valuable rights to our product candidates, our intellectual property, future revenue streams or grant licenses on terms that are not favorable to us.

The estimates and judgments we make, or the assumptions on which we rely, in preparing our consolidated financial statements and condensed consolidated financial statements could prove inaccurate.

Our consolidated financial statements and condensed consolidated financial statements are prepared in accordance with accounting principles generally accepted in the U.S. (the "U.S. GAAP") The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. Such estimates and judgments include revenue recognition, inventory, valuation of stock-based awards, research and development expenses and income tax. We base our estimates on historical experience, facts and circumstances known to us and on various other assumptions that we believe to be reasonable under the circumstances. We cannot provide assurances, however, that our estimates, or the assumptions underlying them, will not change over time or otherwise prove inaccurate. If this is the case, we may be required to restate our consolidated financial statements or condensed consolidated financial statements, which could, in turn, subject us to securities class action litigation.

Defending against such potential litigation relating to a restatement of our consolidated financial statements or condensed consolidated financial statements would be expensive and would require significant attention and resources of our management. Moreover, our insurance to cover our obligations with respect to the ultimate resolution of any such litigation may be inadequate. As a result of these factors, any such potential litigation could have a material adverse effect on our financial results and cause our stock price to decline, which could in turn subject us to securities class action litigation.

Risks Related to Our Common Stock

Our stock price is volatile and may fluctuate due to factors beyond our control.

The market prices for and trading volumes of securities of biotechnology companies, including our securities, have historically been volatile. Our stock has had significant swings in trading prices, in particular in connection with our public communications regarding feedback received from regulatory authorities. For example, over the last 12 months, as of the date of this report, our stock has increased as much as 30% in a single day or decreased as much as 8% in a single day. The market has from time to time experienced significant price and volume fluctuations unrelated to the operating performance of particular companies. The market price of our common stock may fluctuate significantly due to a variety of factors, including but not limited to:

- the commercial performance of our products in the U.S.;
- the timing of our submissions to regulatory authorities and regulatory decisions and developments;
- positive or negative clinical trial results or regulatory interpretations of data collected in clinical trials conducted by us, our strategic partners, our competitors or other companies with investigational drugs targeting the same, similar or related diseases to those targeted by us;
- delays in beginning and completing pre-clinical and clinical trials for potential product candidates;
- delays in entering or failing to enter into strategic relationships with respect to development and/or commercialization of our products or product candidates or entry into strategic relationships on terms that are not deemed to be favorable to us;
- technological innovations, product development or additional commercial product introductions by ourselves or competitors;
- changes in applicable government regulations or regulatory requirements in the approval process;
- developments concerning proprietary rights, including patents and patent litigation matters, such as developments in the interferences declared by the USPTO, including in the near term any outcomes of ongoing interference proceedings and over the longer term the outcomes from any related appeals;
- public concern relating to the commercial value, efficacy or safety of any of our products;
- our ability to obtain funds, through the issuance of equity or equity linked securities or incurrence of debt, or other corporate transactions;
- comments by securities analysts;
- developments in litigation against us;
- changes in senior management; or
- general market conditions in our industry or in the economy as a whole.

Broad market and industry factors may seriously affect the market price of a company's stock, including ours, regardless of actual operating performance. For example, the trading prices of biopharmaceutical companies have been highly volatile as a result of inflation and increased interest rates and overall market volatility. In addition, our operations and performance may be affected by political or civil unrest or military action, including the ongoing conflict between Russia and Ukraine. Additionally, in the past, following periods of volatility in the overall market and the market price of a particular company's securities, securities class action litigation has often been instituted against these companies. Such litigation could result in substantial costs and a diversion of our management's attention and resources.

Our revenues and operating results could fluctuate significantly, which may adversely affect our stock price and our ability to maintain profitability.

Our revenues and operating results may vary significantly from year-to-year and quarter-to-quarter as well as in comparison to the corresponding quarter of the preceding year. Variations may result from one or more factors, including, without limitation:

- timing of purchase orders;
- changes in coverage and reimbursement policies of health plans and other health insurers, especially in relation to those products that are currently manufactured, under development or identified for future development by us;
- re-authorizations processes that may be required for patients who initially obtained coverage by third parties, including government payors, managed care organizations and private health insurers;
- transition from temporary billing codes established by the CMS to permanent medical codes;
- timing of approval of applications filed with the FDA;
- timing of product launches and market acceptance of products launched;
- changes in the amounts spent to research, develop, acquire, license or promote new and existing products;
- results of clinical trial programs;
- serious or unexpected health or safety concerns with our product or product candidates and any resulting clinical holds;
- introduction of new products by others that render one or more of our products obsolete or noncompetitive;
- the ability to maintain selling prices and gross margins on our products;
- increases in the cost of raw materials contained within our products and product candidates;
- manufacturing and supply interruptions, including product rejections or recalls due to failure to comply with manufacturing specifications;
- timing of revenue recognition relating to our distribution agreements;
- changes in estimates or potential asset impairments;
- the ability to protect our intellectual property from being acquired by other entities;
- the ability to avoid infringing the intellectual property of others;
- the impact of the COVID-19 or similar pandemics; and
- the addition or loss of customers.

In addition, in one or more future periods, our results of operations may fall below the expectations of securities analysts and investors. In that event, the market price of our common stock could decline.

Provisions of our certificate of incorporation, bylaws and Delaware law might deter acquisition bids for us that might be considered favorable and prevent or frustrate any attempt to replace or remove the then-current management and board of directors.

Certain provisions of our certificate of incorporation and bylaws may make it more difficult for a third party to acquire control of us or effect a change in our board of directors and management. These provisions include:

- when the board is comprised of six or more directors, classification of our board of directors into two classes, with one class elected each year;
- directors may only be removed for cause by the affirmative vote of a majority of the voting power of all the then-outstanding shares of voting stock;
- prohibition of cumulative voting of shares in the election of directors;
- right of the board of directors to elect directors to fill a vacancy created by the expansion of the board of directors or the resignation, death, disqualification or removal of a director;
- express authorization of the board of directors to make, alter or repeal our bylaws;

- prohibition on stockholder action by written consent;
- advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at stockholder meetings;
- the ability of our board of directors to authorize the issuance of undesignated preferred stock, the terms and rights of which may be established and shares of which may be issued without stockholder approval, including rights superior to the rights of the holders of common stock; and
- a super-majority (66 2/3%) of the voting power of all of the then-outstanding shares of capital stock are required to amend, rescind, alter or repeal our bylaws and certain provisions of our certificate of incorporation.

In addition, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These and other provisions in our certificate of incorporation and our bylaws and in the Delaware General Corporation Law could make it more difficult for stockholders or potential acquirers to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors.

A significant number of shares of our common stock are issuable pursuant to outstanding stock awards, and we expect to issue additional stock awards and shares of common stock to attract and retain employees, directors and consultants. We may also issue shares of common stock to finance our operations and in connection with our strategic goals. The vesting and exercise of these awards and sales of shares will dilute the interests of existing security holders and may depress the price of our common stock.

Currently, our Amended and Restated Certificate of Incorporation authorizes the issuance of up to 198.0 million shares of common stock. As of December 31, 2024, there were approximately 96.9 million shares of common stock outstanding and outstanding awards to purchase 11.6 million shares of common stock under various incentive stock plans. Additionally, as of December 31, 2024, there were approximately 3.4 million shares of common stock available for future issuance under our 2018 Equity Incentive Plan, approximately 0.2 million shares of common stock available for issuance under our Amended and Restated 2013 Employee Stock Purchase Plan, and approximately 1.0 million shares of common stock available for issuance under our 2024 Employment Commencement Incentive Plan.

We may issue additional shares to grant equity awards to our employees, officers, directors and consultants under our 2018 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2024 Employment Commencement Incentive Plan. We may also issue additional common stock and warrants from time to time to finance our operations and in connection with strategic transactions, such as acquisitions and licensing. For example, in February 2020, we issued and sold 2,522,227 shares of common stock to Roche Finance in connection with the entry into the collaboration agreement with Roche.

The issuance of additional shares of common stock or warrants to purchase common stock and the perception that such issuances may occur or exercise of outstanding warrants or stock options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

Future sales of our common stock in the public market could cause our share price to fall.

Sales of a substantial number of our common stock in the public market, including sales by members of our management or board of directors, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity or equity-related securities.

Risks Related to Our Convertible Senior Notes

Servicing our 1.25% notes due 2027 (the "Notes") requires a significant amount of cash, and we may not have sufficient cash flow to pay our debt.

In September 2022, we issued $1,150.0 million aggregate principal amount of 2027 Notes, pursuant to that certain indenture dated as of September 16, 2022, between us, as issuer, and U.S. Bank National Association, as trustee, including $20.0 million of Notes issued to the Michael A. Chambers Living Trust in a private placement. Our ability to make scheduled payments of the principal of, to pay interest on, or to refinance our indebtedness, including the Notes, depends on our future performance, which is subject to many factors, including, economic, financial, competitive and other, beyond our control. We do not expect our business to be able to generate cash flow from operations in the foreseeable future, sufficient to service our debt and make necessary capital expenditures and we may therefore be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining

additional equity capital on terms that may be onerous or highly dilutive. Our ability to refinance the Notes will depend on the capital markets and our financial condition at such times. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations, and limit our flexibility in planning for and reacting to changes in our business.

We may not have the ability to raise the funds necessary to repurchase the Notes as required upon a fundamental change, and our future debt may contain limitations on our ability to repurchase the Notes.

Holders of the Notes will have the right to require us to repurchase their Notes for cash upon the occurrence of a fundamental change at a fundamental change repurchase price equal to 100% of the principal amount of the Notes to be repurchased, plus accrued and unpaid interest, if any. A fundamental change may also constitute an event of default or prepayment under, and result in the acceleration of the maturity of, our then-existing indebtedness. We cannot assure you that we will have sufficient financial resources, or will be able to arrange financing, to pay the fundamental change repurchase price in cash with respect to any Notes surrendered by holders for repurchase upon a fundamental change. In addition, restrictions under our then existing credit facilities or other indebtedness, if any, may not allow us to repurchase the Notes upon a fundamental change. Our failure to repurchase the Notes upon a fundamental change when required would result in an event of default with respect to the Notes which could, in turn, constitute a default under the terms of our other indebtedness, if any. If the repayment of the related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness and repurchase the Notes.

Capped call transactions entered into in connection with the Notes may impact the value of our common stock.

In connection with the Notes, we entered into capped call transactions (the "Capped Call Transactions") with certain financial institutions. The Capped Call Transactions are expected to generally reduce the potential dilution upon conversion of the Notes into shares of our common stock.

In connection with establishing their initial hedges of the Capped Call Transactions, these financial institutions or their respective affiliates may have entered into various derivative transactions with respect to our common stock and/or purchased our common stock. The financial institutions, or their respective affiliates, may modify their hedge positions by entering into or unwinding various derivatives with respect to our common stock and/or purchasing or selling our common stock or other securities of ours in secondary market transactions prior to the maturity of the Notes. This activity may have an impact on the value of our common stock.

General Risks

Unfavorable and uncertain global economic conditions could harm our business, financial condition or results of operations.

Our results of operations could be harmed by general conditions in the global economy and in the global financial markets. A severe or prolonged economic downturn, including the impact of increased interest rates and inflation (such as the recent rise in inflation in the US), could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. Significant uncertainty regarding general political and geopolitical conditions, as well as the stability of financial markets related to any future changes in policies, could adversely impact our business. In addition, a weak or declining economy could strain our manufacturers, possibly resulting in manufacturing disruption, or cause delays in payments for our services by third-party payors or our future collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could harm our business.

We may be subject to product liability claims and our insurance may not be adequate to cover damages.

The current and future use of our product candidates by us and our collaborators in clinical trials, EAPs, the sale of our products, or the use of our products under emergency use vehicles may expose us to liability claims inherent to the manufacture, clinical testing, marketing and sale of medical products. These claims might be made directly by consumers or healthcare providers or indirectly by pharmaceutical companies, our collaborators or others selling such products. Regardless of merit or eventual outcome, we may experience financial losses in the future due to such product liability claims. We have obtained commercial general liability insurance coverage for our clinical trials and the sale of commercial products. However, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against all losses. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

We have expanded, and may continue to expand, our organization and may experience difficulties in managing this growth, which could disrupt our operations.

To support the expansion of our business activities, we have expanded, and may continue to expand, our full-time employee base, as well as our consultant and contractor base. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. Our ability to manage our growth properly and maintain compliance with all applicable rules and regulations will require us to continue to improve our operational, legal, financial and management controls, as well as our reporting systems and procedures. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy.

Our sales and operations are subject to the risks of doing business internationally.

We are increasing our presence in international markets, including emerging markets, subjecting us to many risks that could adversely affect our business and revenues, such as:

- the inability to obtain necessary foreign regulatory or pricing approvals of products in a timely manner;
- uncertainties regarding the collectability of accounts receivable;
- fluctuations in foreign currency exchange rates that may adversely impact our revenues, net income and value of certain of our investments;
- difficulties in staffing and managing international operations;
- the imposition of governmental controls;
- less favorable intellectual property or other applicable laws;
- increasingly complex standards for complying with foreign laws and regulations that may differ substantially from country to country and may conflict with corresponding U.S. laws and regulations;
- the far-reaching anti-bribery and anti-corruption legislation in the UK, including the UK Bribery Act 2010, and elsewhere and escalation of investigations and prosecutions pursuant to such laws;
- compliance with complex import and export control laws;
- restrictions on direct investments by foreign entities and trade restrictions; and
- changes in tax laws and tariffs.

In addition, our international operations are subject to regulation under U.S. law. For example, the Foreign Corrupt Practices Act (“FCPA”) prohibits U.S. companies and their representatives from paying, offering to pay, promising to pay or authorizing the payment of anything of value to any foreign government official, government staff member, political party or political candidate for the purpose of obtaining or retaining business or to otherwise obtain favorable treatment or influence a person working in an official capacity. In many countries, the healthcare professionals we regularly interact with may meet the FCPA's definition of a foreign government official. Failure to comply with domestic or foreign laws could result in various adverse consequences, including: possible delay in approval or refusal to approve a product, recalls, seizures or withdrawal of an approved product from the market, disruption in the supply or availability of our products or suspension of export or import privileges, the imposition of civil or criminal sanctions, the prosecution of executives overseeing our international operations and damage to our reputation. Any significant impairment of our ability to sell products outside of the U.S. could adversely impact our business and financial results.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cybersecurity incidents, could harm our ability to operate our business effectively.

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, our proprietary business information and that of our suppliers, as well as personally identifiable information of the patients using our commercially approved products, clinical trial participants and employees. Similarly, our third-party providers possess certain of our sensitive data. The secure maintenance of this information is critical to our operations and business strategy. Our ongoing operating activities also depend on functioning computer systems. Cyberattacks have increased in frequency and potential harm over time, and the methods used to gain unauthorized access constantly evolve, making it increasingly difficult to anticipate, prevent, and/or detect incidents

successfully in every instance. We are required to expend significant resources in an effort to protect against security incidents and may be required or choose to spend additional resources or modify our business activities, particularly where required by applicable data privacy and security laws or regulations or industry standards. Our security measures may be insufficient, and our information technology and infrastructure, as well as that of our vendors, contractors, and other third-party partners who process information on our behalf or have access to our systems, may be susceptible to security incidents, disruptions, cyberattacks, ransomware, breaches, viruses, phishing attacks and other forms of social engineering, denial-of-service attacks, third-party or employee theft or misuse and other negligent actions. Any such breach could result in a material compromise of our networks, and the information stored there could be accessed, publicly disclosed, lost, stolen, or rendered, permanently or temporarily, inaccessible. Any perceived or actual unauthorized or inadvertent disclosure of personal or other confidential information, cyberattack, or other breach or theft of information could have a material impact on our business, operations or financial results. Any such access, disclosure or other loss of information, including our data being breached at third party providers, could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations and damage our reputation, which could adversely affect our business.

We may incur substantial costs in connection with litigation and other disputes.

In the ordinary course of business we may, and in some cases have, become involved in lawsuits and other disputes such as securities claims, intellectual property challenges, including interferences declared by the USPTO, contractual disputes, and employee matters. We may expend significant amounts of money and company resources in connection with these disputes and it is possible that we may not prevail in claims made against us in such disputes. The outcome of such lawsuits and disputes is inherently uncertain and may have a negative impact on our business, financial condition and results of operations.

The increasing use of social media platforms and artificial intelligence tools presents new risks and challenges.

Social media is increasingly being used to communicate about our products, technologies and programs, and the diseases our product and product candidates are designed to treat. Social media practices in the biopharmaceutical industry continue to evolve and regulations relating to such use are not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media channels to comment on the effectiveness of a product or to report an alleged adverse event. When such disclosures occur, there is a risk that we fail to monitor and comply with applicable adverse event reporting obligations or we may not be able to defend ourselves or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our product and/or product candidates.

Additionally, AI tools are increasingly being used in our industry. We are evaluating, and will continue to evaluate, the use of AI tools throughout our organization. There are risks involved in developing and using AI in our operations, including related to enhanced governmental or regulatory scrutiny and our development and use of AI may not be beneficial to our business, including the development of our product candidates or our profitability or efficiency.

In addition, any misuse of social media or AI may result in inappropriate disclosure of sensitive information or cause reputational harm, give rise to liability, lead to the loss of trade secrets and other IP, or lead to other consequences. If any of these events described above were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face overly restrictive regulatory actions or incur other harm to our business.

We or the third parties upon whom we depend may be adversely affected by natural disasters and/or terrorism attacks, and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage, terrorism attack or other event occurred that prevented us from using all or a significant portion of our office, manufacturing and/or lab spaces, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time.

The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

Item 1B. Unresolved Staff Comments.

None.

Item 1C. Cybersecurity

Program Details

Our information security program is developed using industry standards and best practices as a guide, including the National Institute of Standards and Technology ("NIST") Cybersecurity Framework. The program includes regular internal evaluations, including annual penetration tests and monthly vulnerability scans, as well as evaluations by external vendor partners in support of our operations model. The results of these evaluations are regularly shared with senior management and the Audit Committee of the Board of Directors (the "Audit Committee"), where appropriate.

We have developed and implemented a cybersecurity risk management program intended to protect the Confidentiality, Integrity, and Availability ("CIA") of our critical systems and information.

Our cybersecurity risk management program is integrated into our overall enterprise risk management processes and shares common methodologies, reporting channels and governance processes that apply across the enterprise risk management program to other legal, compliance, strategic, operational, and financial risk areas.

Our cybersecurity risk management program includes:

- A layered defense approach with controls deployed that seek to meet the requirements of the NIST Cybersecurity Framework.
- Risk assessments designed to help identify material cybersecurity risks to our critical systems, information, products, services, and our broader enterprise IT environment.
- A security team principally responsible for managing (a) our cybersecurity risk assessment processes, (b) our security controls, and (c) our response to cybersecurity incidents.
- The use of external service providers, where appropriate, to assess, test or otherwise assist with aspects of our security controls as part of our operational security model.
- A threat intelligence function that informs our cybersecurity and IT personnel about new vulnerabilities and risks that require timely intervention or remediation.
- Cybersecurity awareness training of our employees, incident response personnel, and senior management.
- A cybersecurity incident response plan that includes procedures for responding to cybersecurity incidents.
- A third-party risk management process for service providers, suppliers, and vendors.

As of the date of this Annual Report, we have not experienced any material cybersecurity incidents, but we cannot provide assurance that we will not be materially affected in the future by such risks or any future material incidents.

Oversight

The Audit Committee oversees our information technology systems and related cybersecurity program. Our cybersecurity program is managed by our dedicated Chief Information Security Officer (the "CISO"), reporting directly to the Company's Chief Information Officer (the "CIO"), whose team is responsible for leading the Company's cybersecurity policies and procedures.

Our CIO has over 25 years of experience and has served in a variety of information systems leadership roles in the life sciences industry supporting research and development, commercial sales and marketing, finance, human resources and other corporate functions, and IT architecture, strategy, and planning.

Our CISO has over 20 years of experience, including experience in creating and managing corporate-wide information technology, information/cybersecurity, compliance, privacy, and risk management programs as well as having implemented these initiatives across global organizations.

At least annually, but more often as needed, our CIO provides updates on the program to the Audit Committee. The CIO also provides regular updates to members of the Company's senior management team regarding cyber risks, threats and assessments and material cybersecurity developments of the Company's program.

Item 2. Properties.

A description of the facilities we own and/or occupy is included in the following table. We believe that our current facilities in Cambridge, Andover, Burlington and Bedford, Massachusetts, Columbus, Ohio and Durham, North Carolina are suitable and will provide sufficient capacity to meet the projected needs of our business for the next 12 months. Except as noted below, all of our properties are currently being used in the operation of our business.

Location of Property	Square Footage	Lease Expiration Date	Purpose	Other Information
215 First Street, Cambridge, MA - 1st & 2nd Floor	32,314	September 2025	Laboratory and office space	Corporate headquarters
215 First Street, Cambridge, MA - 4th Floor & Basement	79,048	May 2031	Laboratory and office space	Corporate headquarters
600 Federal Street, Andover, MA	11,832	December 2026	Laboratory and office space	Laboratory and office space
100 Federal Street, Andover, MA	65,589	N/A- facility is owned	Laboratory and office space	Primarily laboratory space
300 Federal Street, Andover, MA	23,102	December 2025	Office space	Office space
55 Network Drive, Burlington, MA	44,740	June 2025	Laboratory and office space	Primarily laboratory space
50-52 Crosby Drive, Bedford, MA	288,000	January 2038	Laboratory and office space	Primarily laboratory space
3435 Stelzer Road, Columbus, OH	151,661	December 2036	Laboratory and office space	Primarily laboratory space
701 West Main Street, Suite 102, Durham, NC	4,346	March 2025	Laboratory and office space	Primarily laboratory space
701 West Main Street, Suite Lab 2806 & 2802, Durham, NC	840	March 2025	Laboratory and office space	Primarily laboratory space

Item 3. Legal Proceedings.

For material legal proceedings, please read *Note 22, Commitments and Contingencies - Litigation* to our consolidated financial statements included in this Annual Report.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information

Our common stock is quoted on the Nasdaq Global Select Market under the same symbol "SRPT".

Holders

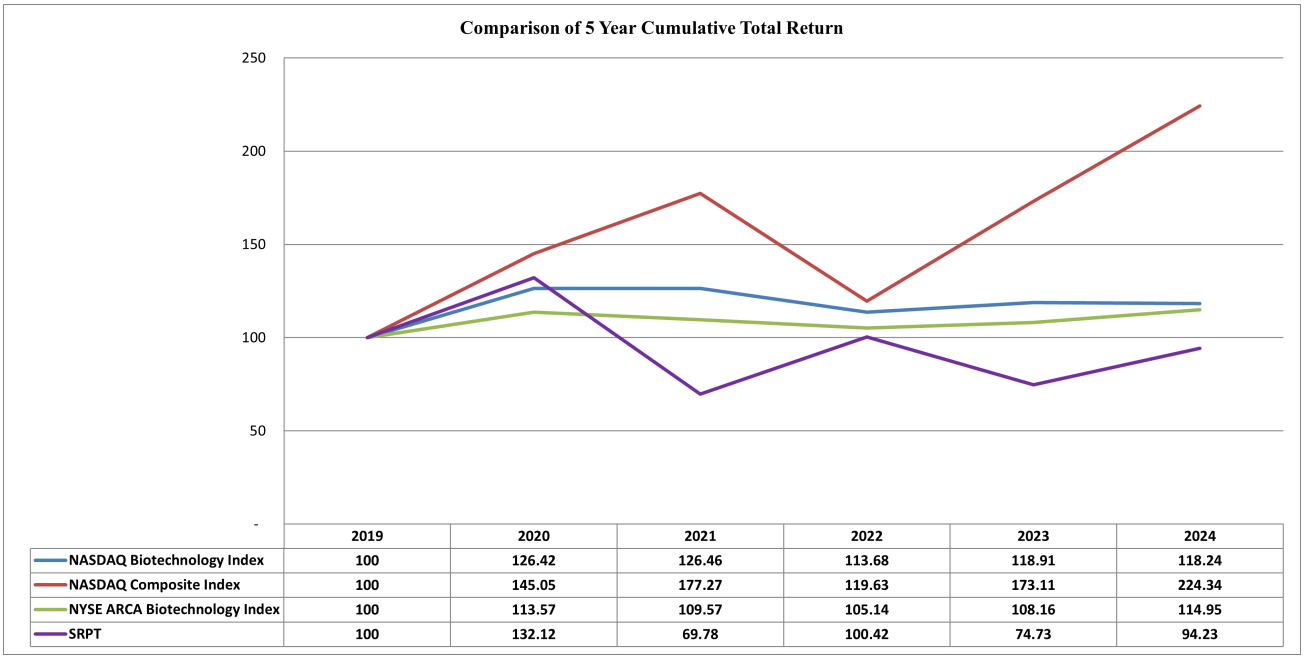
As of February 24, 2025, we had 144 stockholders of record of our common stock.

Dividends

We did not declare or pay cash dividends on our common stock in 2024, 2023 or 2022. We currently expect to retain future earnings, if any, to finance the operation and expansion of our business, and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination related to our dividend policy will be made at the discretion of our board of directors.

Performance Graph

The following graph compares the performance of our Common Stock for the periods indicated with the performance of the NASDAQ Composite Index, NASDAQ Biotechnology Index and the NYSE ARCA Biotechnology Index. This graph assumes an investment of $100 after the market closed December 31, 2019 in each of our common stock, the NASDAQ Composite Index, NASDAQ Biotechnology Index and the NYSE ARCA Biotechnology Index, and assumes reinvestment of dividends, if any. The stock price performance shown on the graph below is not necessarily indicative of future stock price performance. This graph is not "soliciting material," is not deemed "filed" with the SEC and is not to be incorporated by reference into any of our filings under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date hereof and irrespective of any general incorporation language in any such filing.



	2019	2020	2021	2022	2023	2024
NASDAQ Biotechnology Index	100	126.42	126.46	113.68	118.91	118.24
NASDAQ Composite Index	100	145.05	177.27	119.63	173.11	224.34
NYSE ARCA Biotechnology Index	100	113.57	109.57	105.14	108.16	114.95
SRPT	100	132.12	69.78	100.42	74.73	94.23

Recent Sales of Unregistered Securities.

None.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers.

None.

Item 6. Reserved

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The purpose of Management's Discussion and Analysis of Financial Condition and Results of Operations is to provide an understanding of the financial condition, changes in financial condition and results of operations of Sarepta Therapeutics, Inc. The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and related notes included elsewhere in this Annual Report on Form 10-K. This discussion contains forward-looking statements that involve risks and uncertainties. Please review our legend titled "Forward-Looking Information" at the beginning of this Annual Report on Form 10-K which is incorporated herein by reference. Our actual results could differ materially from those discussed below. Factors that could cause or contribute to such differences include, but are not limited to, those identified below, and those discussed in the section titled "Risk Factors" included elsewhere in this Annual Report on Form 10-K. Throughout this discussion, unless the context specifies or implies otherwise, the terms "Sarepta", "we", "us" and "our" refer to Sarepta Therapeutics, Inc. and its subsidiaries.

This section discusses 2024 and 2023 items and year-to-year comparisons between 2024 and 2023. Discussions of 2022 items and year-to-year comparisons between 2023 and 2022 have been excluded from this Form 10-K and can be found in "Management's Discussion and Analysis of Financial Condition and Results of Operations" in Part II, Item 7 of our Annual Report on Form 10-K for the fiscal year ended December 31, 2023.

Overview

We are a commercial-stage biopharmaceutical company focused on helping patients through the discovery and development of unique RNA-targeted therapeutics, gene therapy and other genetic therapeutic modalities for the treatment of rare diseases. Applying our proprietary, highly differentiated and innovative technologies, and through collaborations with our strategic partners, we have developed multiple approved products for the treatment of Duchenne and are developing potential therapeutic candidates for a broad range of diseases and disorders, including Duchenne, LGMDs, and other neuromuscular and central nervous system related disorders.

We commercialized four products that have been approved by the FDA:

- The PMO Products:
  - EXONDYS 51 (eteplirsen) Injection ("EXONDYS 51"), granted accelerated approval by the FDA on September 19, 2016, is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 51 skipping. EXONDYS 51 uses our PMO chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene.
  - VYONDYS 53 (golodirsen) Injection ("VYONDYS 53"), granted accelerated approval by the FDA on December 12, 2019, is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 53 skipping. VYONDYS 53 uses our PMO chemistry and exon-skipping technology to skip exon 53 of the dystrophin gene.
  - AMONDYS 45 (casimersen) Injection ("AMONDYS 45"), granted accelerated approval by the FDA on February 25, 2021, is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 45 skipping. AMONDYS 45 uses our PMO chemistry and exon-skipping technology to skip exon 45 of the dystrophin gene.
- ELEVIDYS (delandistrogene moxeparvovec-rokl), approved by the FDA on June 20, 2024, is an AAV-based gene therapy for the treatment of ambulatory patients at least four years old with Duchenne with a confirmed mutation in the Duchenne gene. ELEVIDYS is also approved for non-ambulatory patients under the accelerated approval pathway. ELEVIDYS was previously granted accelerated approval by the FDA on June 22, 2023 for the treatment of ambulatory patients aged four through five years with Duchenne with a confirmed mutation in the Duchenne gene. ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the Duchenne gene.

We are in the process of conducting various clinical trials for our approved products, including studies that are required to comply with our post-marketing FDA requirements/commitments to verify and describe the clinical benefit of these products.

A summary description of our key product candidates, including those in collaboration with our strategic partners, is as follows:

- *SRP-9003 (LGMD, gene therapy program)*. We are developing gene therapy programs for various forms of LGMD. The most advanced of our LGMD product candidates, SRP-9003, aims to treat LGMD2E, also known as beta-sarcoglycanopathy, a severe and debilitating form of LGMD characterized by progressive muscle fiber loss, inflammation and muscle fiber replacement with fat and fibrotic tissue. SRP-9003 is designed to transfect a gene that codes for and restores beta-sarcoglycan protein with the goal of restoring the dystrophin associated protein complex. SRP-9003 has generated positive pre-clinical safety and efficacy data utilizing the AAVrh.74 vector, the same vector used in our SRP-9001 gene therapy program. A Phase 1/2a trial of SRP-9003 commenced in the fourth quarter of 2018. In June 2020, we announced safety and expression results from three clinical trial participants in the high-dose cohort measured at 60 days, and one-year functional data from three clinical trial participants in the low-dose cohort. In March 2022, we announced 36-month functional data from three clinical trial participants in the low-dose cohort and 24-month functional data from two clinical trial participants in the high-dose cohort. In December 2024, we announced that we had completed enrollment and dosing in EMERGENE (Study SRP-9003-301), a Phase 3 clinical trial of SRP-9003 (bidridistrogene xeboparvovec).

Our pipeline includes programs in various stages of pre-clinical and clinical development, reflecting our multifaceted approach and expertise in precision genetic medicine to make a profound difference in the lives of patients suffering from rare diseases.

We have developed proprietary state-of-the-art CMC and manufacturing capabilities that allow synthesis and purification of our products and product candidates to support both clinical development as well as commercialization. Our current main focus in manufacturing is to sustain large-scale production of our PMO-based therapies and optimizing manufacturing for gene therapy-based product candidates. We have entered into certain manufacturing and supply arrangements with third-party suppliers and will utilize these capabilities to support production of certain of our products and product candidates and their components. In 2017, we opened a facility in Andover, Massachusetts, which significantly enhanced our research and development manufacturing capabilities. However, we currently do not have internal large scale GMP manufacturing capabilities to produce our products and product candidates for commercial and/or clinical use.

The likelihood of our long-term success must be considered in light of the expenses, difficulties and delays frequently encountered in the development and commercialization of new pharmaceutical products, competitive factors in the marketplace, the risks associated with government sponsored reimbursement programs and the complex regulatory environment in which we operate.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations is based upon our consolidated financial statements included elsewhere in this Annual Report on Form 10-K. The preparation of our consolidated financial statements in accordance with U.S. GAAP requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities for the periods presented. Some of these judgments can be subjective and complex, and, consequently, actual results may differ from these estimates. We believe that the estimates and judgments upon which we rely are reasonable based upon historical experience and information available to us at the time that we make these estimates and judgments. To the extent there are material differences between these estimates and actual results, our consolidated financial statements will be affected. Although we believe that our judgments and estimates are appropriate, actual results may differ from these estimates. We believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our consolidated financial statements:

- inventory; and
- income tax.

Inventory Valuation

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis. We capitalize inventory costs associated with products following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. EXONDYS 51, VYONDYS 53, AMONDYS 45 and ELEVIDYS inventory that may be used in clinical development programs is charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes.

We periodically analyze our inventories for excess amounts or obsolescence and write down obsolete or otherwise unmarketable inventory to its estimated net realizable value based on assumptions about expected future demand and market conditions. Additionally, though our products are subject to strict quality control and monitoring, which we perform throughout the manufacturing processes, certain batches or units of product may not meet quality specifications. Expense incurred related to excess

inventory, obsolete inventory, or inventories that do not meet our quality specifications is recorded as a component of cost of sales in the consolidated statements of comprehensive income (loss).

Income Tax

We follow the asset and liability method of accounting for income taxes, which requires the recognition of deferred tax assets and liabilities for expected future tax consequences attributable to differences between the consolidated financial statement carrying amounts of existing assets and liabilities and their respective tax bases. Deferred tax assets and liabilities are measured using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance is recorded to reduce the net deferred tax asset to zero when it is more likely than not that the net deferred tax asset will not be realized. As of December 31, 2024, we continued to maintain a full valuation allowance against all of our deferred tax assets, with the exception of deferred tax assets in certain foreign jurisdictions, based on management's evaluation of all available evidence, including our earnings history.

We will continue to monitor the realizability of our deferred tax assets in future periods. We may release all or a portion of the valuation allowance in the near-term; however, the release of the valuation allowance, as well as the exact timing and the amount of such release, continue to be subject to, among other things, our level of profitability, revenue growth and expectations regarding future profitability. If and when we determine the valuation allowance should be released or reduced, the adjustment would result in a benefit to income tax expense for the period the release is recorded, which could have a material impact on net earnings.

We recognize the effect of income tax positions only if those positions are more likely than not of being sustained upon an examination. The calculation of our tax liabilities (or amount of reduction in our deferred tax assets from net operating loss carryover and research credit carryover) resulting from uncertain tax positions can involve significant judgment. Further, the calculation may involve the application of complex tax regulations in a foreign jurisdiction. Any significant impact as a result of changes in underlying facts, law, tax rates, tax audit, or review could lead to adjustments to our deferred tax asset, income tax expense, our effective tax rate, and/or our cash flow. Although we believe that we have adequately provided for tax liabilities resulting from uncertain tax positions, the actual amounts paid, if any, could have a material impact on our results of operations. Interest and penalties associated with uncertain tax positions are classified as a component of income tax expense.

Please read *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* to the consolidated financial statements, included elsewhere in this Annual Report on Form 10-K, for a further discussion of our critical accounting policies and estimates.

The following table sets forth selected consolidated statements of income (loss) data for each of the periods indicated:

	For the Year Ended December 31,			
	2024	2023	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Products, net	$ 1,787,960	$ 1,144,876	$ 643,084	56%
Collaboration and other	114,019	98,460	15,559	16%
Total revenues	1,901,979	1,243,336	658,643	53%
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	319,099	150,343	168,756	112%
Research and development	804,522	877,387	(72,865)	(8)%
Selling, general and administrative	557,872	481,871	76,001	16%
Amortization of in-licensed rights	2,405	1,559	846	54%
Total cost and expenses	1,683,898	1,511,160	172,738	11%
Operating income (loss)	218,081	(267,824)	485,905	NM*
Other income (loss), net:				
Other income, net	42,693	33,055	9,638	29%
Loss on debt extinguishment	—	(387,329)	387,329	(100)%
Gain from sale of Priority Review Voucher	—	102,000	(102,000)	(100)%
Total other income (loss), net	42,693	(252,274)	294,967	NM*
Income (loss) before income tax expense	260,774	(520,098)	780,872	NM*
Income tax expense	25,535	15,879	9,656	61%
Net income (loss)	$ 235,239	$ (535,977)	$ 771,216	NM*
Earnings (loss) per share:				
Basic	$ 2.47	$ (5.80)	$ 8.27	NM*
Diluted	$ 2.34	$ (5.80)	$ 8.14	NM*

* NM: not meaningful

Revenues

The following table summarizes the components of our net product revenues, by product, for the periods indicated:

	For the Year Ended December 31,			
	2024	2023	Change	Change
	(in thousands)		$	%
PMO Products	$ 967,169	$ 944,520	$ 22,649	2%
ELEVIDYS	820,791	200,356	620,435	NM*
Products, net	$ 1,787,960	$ 1,144,876	$ 643,084	56%

* NM: not meaningful

Net product revenues for our products for 2024 increased by $643.1 million, or 56%, compared with 2023. The increase primarily reflects an increase in net product revenues of ELEVIDYS of $620.4 million in 2024 as a result of its initial FDA approval in June 2023 and subsequent expanded label approval in June 2024.

The following table summarizes the components of our collaboration and other revenues for the periods indicated:

	For the Year Ended December 31,			
	2024	2023	Change	Change
	(in thousands)		$	%
Contract manufacturing	$ 49,038	$ 9,216	$ 39,822	NM*
Amortization of performance obligations**	48,000	89,244	(41,244)	(46)%
Royalty revenue	16,981	—	16,981	NM*
Total collaboration and other	$ 114,019	$ 98,460	$ 15,559	16%

* NM: not meaningful

** Related to the recognition of previously deferred revenue under the Roche collaboration agreement as the Company satisfies its performance obligations under the contract. For more information, please read *Note 3, License and Collaboration Agreements*.

Collaboration and other revenues relate to our collaboration arrangement with Roche. For 2024 and 2023, we recognized $114.0 million and $98.5 million of collaboration and other revenues, respectively. In accordance with the Roche Agreement, the parties agreed to enter into a supply agreement in order for us to supply Roche with clinical and commercial batches of ELEVIDYS (the "Supply Agreement"). Roche utilizes the supply for sales of ELEVIDYS in territories outside of the U.S where Roche has received certain approvals for ELEVIDYS. We are eligible to receive royalties on these sales. While the Supply Agreement is in the process of being negotiated, we delivered batches of commercial ELEVIDYS supply to Roche that were agreed upon on a purchase order-by-purchase order basis. For 2024 and 2023, we recognized $49.0 million and $9.2 million of contract manufacturing revenue, respectively, which is related to these Roche shipments. In addition, we recognized $17.0 million of royalty revenue from sales of ELEVIDYS by Roche in 2024, with no similar activity for 2023. For 2024, we recognized $48.0 million in collaboration revenue related to Roche's declined option to acquire the ex-US rights to a certain external, early-stage Duchenne development program, as compared to the $89.2 million in collaboration revenue in 2023 related to the amortization of the single, combined performance obligation under the Roche Agreement, which was fully amortized as of December 31, 2023. Please refer to *Note 3, License and Collaboration Agreements* for further discussion of the Roche Agreement.

Cost of sales (excluding amortization of in-licensed rights)

Our cost of sales (excluding amortization of in-licensed rights) consists of inventory costs that relate to sales of our products and the related overhead costs and royalty payments primarily to BioMarin and UWA for our PMO Products and to Nationwide for ELEVIDYS. Prior to receiving regulatory approval for our products, we expensed manufacturing and material costs as research and development expenses. For the PMO Products, all previously expensed manufacturing costs had been fully consumed prior to 2023. For ELEVIDYS sold in 2024, a portion of related manufacturing costs incurred had previously been expensed as research and development expenses. For ELEVIDYS sold in 2023, the majority of related manufacturing costs incurred had previously been expensed as research and development expenses. If product related costs had not previously been expensed as research and development expenses prior to receiving FDA approval, the incremental inventory costs related to ELEVIDYS sold, including products sold to Roche under the Roche Agreement, would have been approximately $100.8 million and $33.9 million higher for 2024 and 2023, respectively.

The following table summarizes the components of our cost of sales (excluding amortization of in-licensed rights) for the periods indicated:

	For the Year Ended December 31,			
	2024	2023	Change	Change
	(in thousands)		$	%
Inventory costs related to products sold (excluding products sold to Roche**)	$ 249,108	$ 108,988	$ 140,120	129%
Royalty payments	47,744	39,537	8,207	21%
Inventory costs related to products sold to Roche**	22,247	1,818	20,429	NM*
Total cost of sales (excluding amortization of in-licensed rights)	$ 319,099	$ 150,343	$ 168,756	112%

* NM: not meaningful

** See above for further details regarding product supply sold to Roche via contract manufacturing under the Roche Agreement.

The cost of sales (excluding amortization of in-licensed rights) for 2024 increased by $168.8 million, or 112%, compared with 2023. The change primarily reflects an increase in cost of sales related to ELEVIDYS due to an increase in demand following its initial FDA approval in June 2023 and subsequent expanded label approval in June 2024, as well as increases in the write-offs of certain batches of our products not meeting our quality specifications. For 2024 and 2023, we recognized $22.2 million and $1.8 million, respectively, of cost of sales related to products sold to Roche under the Roche Agreement.

Research and development expenses

Research and development expenses consist of costs associated with research activities as well as those associated with our product development efforts, conducting pre-clinical trials, clinical trials and manufacturing activities. Direct research and development expenses associated with our programs include clinical trial site costs, clinical manufacturing costs, costs incurred for consultants, up-front fees and milestones paid to third parties in connection with technologies that have not reached technological feasibility and do not have an alternative future use, and other external services, such as data management and statistical analysis support, and materials and supplies used in support of clinical programs. Indirect costs of our programs include salaries, stock-based compensation and allocation of our facility- and technology-related costs.

Research and development expenses represent a substantial percentage of our total operating expenses. We do not maintain or evaluate and, therefore, do not allocate internal research and development costs on a project-by-project basis. As a result, a significant portion of our research and development expenses are not tracked on a project-by-project basis, as the costs may benefit multiple projects.

The following table summarizes our research and development expenses, by project, for each of the periods indicated:

	For the Year Ended December 31,			
	2024	2023	Change	Change
	(in thousands)		$	%
SRP-9001	$ 307,564	$ 282,207	$ 25,357	9%
LGMD platform	99,122	58,529	40,593	69%
Eteplirsen (exon 51)	70,213	90,829	(20,616)	(23)%
Other gene therapies	33,272	29,411	3,861	13%
PPMO platform	31,926	78,231	(46,305)	(59)%
Gene editing	14,853	12,177	2,676	22%
Casimersen (exon 45)	14,805	21,264	(6,459)	(30)%
Golodirsen (exon 53)	10,062	16,556	(6,494)	(39)%
Other projects	9,064	23,520	(14,456)	(61)%
Internal research and development expenses	339,321	370,677	(31,356)	(8)%
Roche collaboration reimbursement	(125,680)	(106,014)	(19,666)	19%
Total research and development expenses	$ 804,522	$ 877,387	$ (72,865)	(8)%

The following table summarizes our research and development expenses by category for each of the periods indicated:

	For the Year Ended December 31,			
	2024	2023	Change	Change
	(in thousands)		$	%
Manufacturing expenses*	$ 329,011	$ 345,826	$ (16,815)	(5)%
Compensation and other personnel expenses	164,322	161,763	2,559	2%
Clinical trial expenses	163,565	187,289	(23,724)	(13)%
Facility- and technology-related expenses	90,697	87,307	3,390	4%
Stock-based compensation	74,010	82,489	(8,479)	(10)%
Professional services	30,640	26,749	3,891	15%
Pre-clinical expenses	6,359	11,838	(5,479)	(46)%
Research and other	71,598	80,140	(8,542)	(11)%
Roche collaboration reimbursement	(125,680)	(106,014)	(19,666)	19%
Total research and development expenses	$ 804,522	$ 877,387	$ (72,865)	(8)%

*Beginning in 2024, we implemented an updated manufacturing absorption methodology that allocates the absorption of indirect manufacturing costs to their respective originating categories. Research and development expenses by category, specifically, manufacturing expenses, compensation and other personnel expenses, facility- and technology-related expenses and professional services, have been reclassified for 2023 for comparability. This reallocation has no impact on the total research and development expenses recognized.

Research and development expenses for 2024 decreased by $72.9 million, or 8%, compared with 2023. The decrease was primarily driven by the following:

- $16.8 million decrease in manufacturing expenses primarily due to the capitalization of commercial batches of ELEVIDYS manufactured upon its approval in June 2023, a decrease in clinical batches for our PPMO platform as a result of our decision to discontinue our PPMO programs during 2024, partially offset by $91.9 million of costs associated with the termination of the development, commercial manufacturing and supply agreement (the "Thermo Agreement") related to Brammer Bio MA, LLC, an affiliate of Thermo Fisher Scientific, Inc. ("Thermo") in August 2024 and an increase in costs associated with our LGMD gene therapy programs. Please refer to *Note 22, Commitments and Contingencies* for further discussion of the Thermo Agreement;
- $2.6 million increase in compensation and other personnel expenses primarily due to changes in headcount, partially offset by an increase in indirect manufacturing costs absorption offset as a result of ELEVIDYS approval in June 2023;
- $23.7 million decrease in clinical trial expenses primarily due to a decrease in activity for our PPMO platform and our decision to discontinue our PPMO programs in November 2024, as well as a ramp-down of the ESSENCE studies for AMONDYS 45 and VYONDYS 53;
- $3.4 million increase in facility- and technology-related expenses primarily due to our continuing expansion efforts, partially offset by an increase in indirect manufacturing costs absorption offset as a result of ELEVIDYS approval in June 2023;
- $8.5 million decrease in stock-based compensation expense primarily due to an increase in indirect manufacturing costs absorption offset as a result of ELEVIDYS approval in June 2023, partially offset by the achievement of performance conditions related to certain restricted stock units with performance conditions ("PSUs") during 2024;
- $3.9 million increase in professional services primarily due to an increase in reliance on third-party research and development contractors for clinical programs, partially offset by an increase in indirect manufacturing costs absorption offset as a result of ELEVIDYS approval in June 2023;
- $5.5 million decrease in pre-clinical expenses primarily due to a decrease in activity in our PPMO platform and decision to discontinue our PPMO programs in November 2024;
- $8.5 million decrease in research and other expenses primarily due to timing of achievement of certain up-front and milestone payments, partially offset by an increase in sponsored research with academic institutions during 2024; and
- $19.7 million increase in the offset to expense associated with a collaboration reimbursement from Roche due to reimbursable costs associated with the termination of the Thermo Agreement during 2024, with no similar activity during 2023. This was partially offset by a decrease in clinical supply costs due to timing for our SRP-9001 gene therapy programs.

Selling, general and administrative expenses

Selling, general and administrative expenses consist of salaries, benefits, stock-based compensation and related costs for personnel in our executive, finance, legal, information technology, business development, human resources, commercial and other general and administrative functions. Other general and administrative expenses include an allocation of our facility- and technology-related costs and professional fees for legal, consulting and accounting services.

The following table summarizes our selling, general and administrative expenses by category for each of the periods indicated:

	For the Year Ended December 31,			
	2024	2023	Change	Change
	(in thousands)		$	%
Professional services	$ 183,505	$ 158,279	$ 25,226	16%
Compensation and other personnel expenses	171,508	157,317	14,191	9%
Stock-based compensation	110,290	100,025	10,265	10%
Facility- and technology-related expenses	50,903	44,090	6,813	15%
Other	43,093	23,031	20,062	87%
Roche collaboration reimbursement	(1,427)	(871)	(556)	64%
Total selling, general and administrative expenses	$ 557,872	$ 481,871	$ 76,001	16%

Selling, general and administrative expenses for 2024 increased by $76.0 million, or 16%, compared with 2023. The increase was primarily driven by the following:

- $25.2 million increase in professional service expenses primarily related to ongoing litigation matters, our continuing expansion efforts and continuing efforts to commercialize ELEVIDYS;
- $14.2 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $10.3 million increase in stock-based compensation expense primarily related to the achievement of performance conditions related to certain PSUs during the year ended December 31, 2024 and changes in headcount;
- $6.8 million increase in facility- and technology-related expenses primarily due to our continuing expansion efforts; and
- $20.1 million increase in other expenses primarily due to the timing of charitable contribution activity.

Amortization of in-licensed rights

Amortization of in-licensed rights relates to the agreements we entered into with UWA, Nationwide, BioMarin and Parent Project Muscular Dystrophy in April 2013, December 2016, July 2017 and May 2018, respectively. Each in-licensed right is being amortized on a straight-line basis over the remaining life of the relevant patent from the date the related fee was incurred, either the regulatory approval or the first commercial sale of the applicable product. For 2024 and 2023, we recorded amortization of in-licensed rights of $2.4 million and $1.6 million, respectively.

Other income (expense), net

Other income (expense), net primarily consists of interest expense on our debt instruments, interest income on our cash, cash equivalents and investments, amortization of investment premium or accretion of investment discount, unrealized gain or loss from our investment in our strategic investments, the changes in the fair value of the derivative assets associated with the capped call options for our convertible senior notes due on November 15, 2024 (the "2024 Notes") and the changes in the fair value of contingent consideration related to regulatory-related contingent payments meeting the definition of a derivative liability. Our cash equivalents and investments consist of money market funds, corporate bonds, government and government agency debt securities and certificates of deposit.

Other income, net for 2024 increased by approximately $9.6 million compared to 2023. The change is primarily due to the impairment of a strategic investment during 2023, with no similar activity during 2024. This change was partially offset by a decrease in interest income and accretion of investment discount, net as a result of lower interest rates and the investment mix of our investment portfolio and an increase in the fair value of derivatives, primarily related to contingent consideration.

Loss on debt extinguishment

On March 2, 2023, we entered into separate, privately negotiated exchange agreements with certain holders of the outstanding 2024 Notes, which resulted in an exchange of $313.5 million in aggregate principal value of the 2024 Notes for approximately 4.5 million shares of our common stock (the "2024 Notes Exchange"). The exchange was not pursuant to the conversion privileges included in the terms of the debt at issuance and, therefore, was accounted for as a debt extinguishment, resulting in a recognition of an extinguishment loss of $387.3 million for 2023. There was no similar activity in 2024.

Gain from sale of Priority Review Voucher

In June 2023, we entered into an agreement to sell the rare pediatric disease Priority Review Voucher ("ELEVIDYS PRV") we received from the FDA in connection with the approval of ELEVIDYS for consideration of $102.0 million, with no commission costs. The transaction was not subject to the conditions set forth under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and closed in June 2023. The net proceeds were recorded as a gain from sale of the ELEVIDYS PRV for 2023 as it did not have a carrying value at the time of the sale. There was no similar activity in 2024.

Income tax expense

Income tax expense for 2024 and 2023 was approximately $25.5 million and $15.9 million, respectively. Income tax expense for 2024 and 2023 relates to state, foreign and federal taxes for which available tax losses or credits were not available to offset. As of December 31, 2024, we continued to maintain a full valuation allowance against our deferred tax assets, with the exception of deferred tax assets in certain foreign jurisdictions. We continue to monitor the available evidence relative to recovery of our deferred tax assets and whether such evidence would be sufficient to conclude that it is more likely than not that such deferred tax assets may be partially or fully recoverable. If we were to remove our valuation allowance in part or full, any such adjustment could have a material impact on our effective tax rate in the applicable period and beyond. Refer to *Note 18, Income Taxes* for discussion of the key drivers impacting our effective tax rate.

Liquidity and Capital Resources

The following table summarizes our financial condition for each of the periods indicated:

	For the Year Ended December 31,			
	2024	2023	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 1,103,010	$ 428,430	$ 674,580	157%
Short-term investments	251,782	1,247,820	(996,038)	(80)%
Non-current investments	133,163	—	133,163	NM*
Restricted cash	15,579	15,579	—	(—)%
Total cash, cash equivalents and investments	$ 1,503,534	$ 1,691,829	$ (188,295)	(11)%
Borrowings:				
Convertible debt	$ 1,137,124	$ 1,237,998	$ (100,874)	(8)%
Total borrowings	$ 1,137,124	$ 1,237,998	$ (100,874)	(8)%
Working capital				
Current assets	$ 3,073,463	$ 2,579,331	$ 494,132	19%
Current liabilities	731,684	653,659	78,025	12%
Total working capital	$ 2,341,779	$ 1,925,672	$ 416,107	22%

For 2024 and 2023, our principal sources of liquidity were primarily derived from sales of our products, net proceeds from sale of the ELEVIDYS PRV, proceeds from the settlement of capped call options associated with the 2024 Notes (the "2017 Capped Calls") and our collaboration arrangement with Roche. Our principal uses of cash are research and development expenses, manufacturing costs, selling, general and administrative expenses, investments, capital expenditures, business development transactions, settlement of long-term debt and other working capital requirements. Refer to *Note 13, Indebtedness* and *Note 19, Leases* for additional discussion of our outstanding indebtedness and material changes to our leasing obligations, respectively. The changes in our working capital primarily reflect the use of cash in operating activities, as well as an increase in inventory due to the capitalization of ELEVIDYS inventory after its approval in June 2023. While our contractual obligations, commitments and debt service requirements over the next several years are significant, we intend to continue to fund our short-term financing needs and working capital requirements from cash flows of operating activities as well as cash on hand and such sources are anticipated to be adequate to fund working capital requirements for at least twelve months from the date these consolidated financial statements were issued.

Beyond 2025, our cash requirements will depend extensively on our ability to advance our research, development and commercialization of product candidates. We may seek additional financings primarily from, but not limited to, the sale and issuance of equity and debt securities, the licensing or sale of our technologies and entering into additional government contracts and/or funded research and development agreements. Our future expenditures and long-term capital requirements may be substantial and will depend on many factors, including but not limited to the following:

- our ability to continue to generate revenues from sales of commercial products and potential future products;
- the timing and costs associated with our expansion efforts;
- the timing and costs associated with repurchases of our common stock under our $500.0 million share repurchase program, approved by our Board of Directors in November 2024 and effective for 18 months;
- the timing and costs of building out our manufacturing capabilities;
- the timing of payments related to our future inventory commitments and manufacturing obligations;

- the timing and costs associated with our existing lease obligations and new obligations expected to be entered into in future years;
- the timing and costs associated with our clinical trials and pre-clinical trials;
- the attainment of milestones and our obligations to make milestone payments to Arrowhead, Myonexus's selling shareholders, BioMarin, Nationwide, UWA and other institutions;
- obligations to holders of our 1.25% convertible senior notes due on September 15, 2027 ("2027 Notes"); and
- the costs of filing, prosecuting, defending and enforcing patent claims and our other intellectual property rights.

We cannot provide assurances that financing will be available when and as needed or that, if available, the financings will be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we require, this would have a material adverse effect on our business and the results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution. We believe that existing cash and cash equivalents, along with future cash generated from operations will be sufficient to meet the capital requirements of our operations for the next 12 months and foreseeable future.

We have entered into long-term contractual arrangements from time to time for our facilities, the provision of goods and services, and issuance of debt securities, among others. Additional information regarding our obligations under debt, lease, and manufacturing arrangements is provided in *Note 13, Indebtedness, Note 19, Leases, Note 22, Commitments and Contingencies* and *Note 23, Subsequent event,* respectively, to the consolidated financial statements. The following table summarizes our total obligations under debt, lease, and manufacturing arrangements:

	As of December 31, 2024		
	Due in less than one year	Due in greater than one year	Total
	(in thousands)		
Debt obligations (1)	$ 14,375	$ 1,178,750	$ 1,193,125
Lease obligations (2)	24,396	328,762	353,158
Manufacturing obligations (3)	943,067	293,434	1,236,501
Total obligations under debt, lease and manufacturing arrangements	$ 981,838	$ 1,800,946	$ 2,782,784

(1) Interest payments are included within the future debt obligations.
(2) Lease obligations only include real estate leases that had commenced prior to December 31, 2024.
(3) The leases embedded in a certain supply agreement are included in manufacturing obligations. The increase in short-term manufacturing commitments is primarily driven by ramp-up of ELEVIDYS manufacturing activities as a result of anticipated increase in demand.

For products and product candidates that are currently approved or are in various research and development stages, we may be obligated to make up to $2.3 billion of future development, regulatory, up-front royalty and sales milestone payments associated with our license and collaboration agreements. Excluded from this metric are $10.3 billion of future development, regulatory and sales milestone payments associated with our license and collaboration agreement with Arrowhead, as the transaction had not closed as of December 31, 2024. When the license and collaboration agreement with Arrowhead became effective in February 2025, we paid Arrowhead an up-front payment of $500.0 million and invested $325.0 million in Arrowhead's common stock at a premium to the valuation on the closing date. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory or commercial milestones. Because the achievement of these milestones is not probable and payment is not required as of December 31, 2024, such contingencies have not been recorded in our consolidated financial statements. Amounts related to contingent milestone payments are not yet considered contractual obligations as they are contingent on the successful achievement of certain development, regulatory approval and commercial milestones.

Cash Flows

The following table summarizes our cash flow activity for each of the periods indicated:

	For the Year Ended December 31,			
	2024	2023	Change	Change
	(in thousands)		$	%
Cash (used in) provided by				
Operating activities	$ (205,787)	$ (500,993)	$ 295,206	(59)%
Investing activities	755,561	(165,803)	921,364	NM*
Financing activities	124,806	125,004	(198)	(—)%
Increase (decrease) in cash and cash equivalents	$ 674,580	$ (541,792)	$ 1,216,372	(225)%

* NM: not meaningful

Operating Activities

Cash used in operating activities, which consists of our net income (loss) adjusted for non-cash items and changes in net operating assets and liabilities, totaled $205.8 million and $501.0 million of cash in 2024 and 2023, respectively. Cash used in operating activities in 2024 was primarily driven by the net income of $235.2 million, adjusted for the following non-cash items:

- $184.3 million in stock-based compensation expense;
- $62.7 million in non-cash termination charges as a result of the Thermo Agreement termination;
- $37.7 million in depreciation and amortization expense;
- $16.2 million reduction in the carrying amount of the right of use assets;
- $7.8 million charge related to the change in the fair value of derivatives; and
- $7.1 million in other non-cash items.

These amounts were partially offset by $40.3 million in accretion of investment discount, net.

The net cash outflow from changes in our operating assets and liabilities was primarily driven by the following:

- $395.2 million increase in inventory primarily due to capitalized inventory related to ELEVIDYS;
- $201.7 million increase in accounts receivable due to an increase in demand for ELEVIDYS following its initial FDA approval in June 2023 and subsequent expanded label approval in June 2024 and an increase in payment terms for product sales related to the PMO Products;
- $188.6 million increase in manufacturing-related deposits and prepaids primarily due to an increase in prepaids for raw materials and batch fees with Catalent, partially offset by decreases in manufacturing-related deposits and prepaids at Thermo as a result of the termination of the Thermo Agreement during 2024; and
- $32.2 million decrease in deferred revenue primarily related to the collaboration with Roche.

These amounts were partially offset by a $110.6 million increase in accounts payable, accrued expenses, lease liabilities and other liabilities primarily due to the timing and invoicing of payments with our CROs and CMOs.

Cash used in operating activities in 2023 was primarily driven by the net loss of $536.0 million, adjusted for the following non-cash items:

- $387.3 million in loss on debt extinguishment of the 2024 Notes;
- $182.5 million in stock-based compensation expense;
- $44.4 million in depreciation and amortization expense;
- $30.3 million in impairments associated with our strategic investments; and
- $19.7 million in other non-cash items.

These amounts were partially offset by the gain of $102.0 million recorded from the sale of the ELEVIDYS PRV and $46.2 million in accretion of investment discount, net.

The net cash outflow from changes in our operating assets and liabilities was primarily driven by the following:

- $185.7 million increase in accounts receivable due to the launch of ELEVIDYS and an increase in the demand of our PMO Products;
- $147.7 million increase in inventory primarily due to capitalized inventory related to ELEVIDYS;
- $86.8 million decrease in deferred revenue primarily related to the collaboration with Roche;
- $50.1 million decrease in accounts payable, accrued expenses, lease liabilities and other liabilities, primarily due to the $54.0 million shortfall payment to Thermo and payments to Catalent for raw materials in 2023 and the overall timing and invoicing of payments; and
- $12.5 million increase in manufacturing-related deposits and prepaids primarily due to the timing and usage of manufacturing prepaids.

Investing Activities

Cash provided by investing activities for 2024 was $755.6 million, while cash used by investing activities for 2023 was $165.8 million. Cash provided by investing activities in 2024 consisted of $2,002.1 million from the maturity and sales of available-for-sale securities, partially offset by purchases of available-for-sale securities, property and equipment and intangible assets of $1,099.6 million, $137.0 million and $10.0 million, respectively.

Cash used in investing activities in 2023 primarily consisted of purchases of available-for-sale securities, property and equipment and intangible assets of $2,044.9 million, $76.1 million and $11.2 million, respectively, partially offset by $1,868.5 million from the maturity and sales of available-for-sale securities and $102.0 million of net proceeds related to the sale of the ELEVIDYS PRV.

Financing Activities

Cash provided by financing activities was $124.8 million in 2024, compared to $125.0 million in 2023. Cash provided by financing activities in 2024 primarily consisted of $79.5 million in proceeds from exercise of options and purchase of stock under our Employee Stock Purchase Program and $45.3 million in proceeds from the settlement of the 2017 Capped Calls.

Cash provided by financing activities in 2023 consisted of $80.6 million in partial settlement the 2017 Capped Calls and $51.2 million in proceeds from exercise of options and purchase of stock under our Employee Stock Purchase Program, partially offset by $6.9 million in third-party debt conversion costs related to the 2024 Notes Exchange.

Other Funding Commitments

We have several on-going clinical trials in various development stages. Our most significant clinical trial expenditures are to CROs. The CRO contracts are generally cancellable at our option. As of December 31, 2024, we had approximately $594.5 million in cancellable future commitments based on existing CRO contracts.

Recent Accounting Pronouncements

Please read *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

Our current investment policy is to maintain a diversified investment portfolio consisting of money market investments, commercial paper, certificates of deposit, government and government agency bonds and high-grade corporate bonds with maturities of 24 months or less. Our cash is primarily deposited in and invested through highly rated financial institutions in the U.S. As of December 31, 2024, we had $1,503.5 million of cash, cash equivalents, restricted cash and investments, comprised of $384.9 million of investments, $1,103.0 million of cash and cash equivalents and $15.6 million of restricted cash. All our debt securities are classified as available-for-sale. The fair value of cash equivalents and investments is subject to change as a result of potential changes in market interest rates. The potential change in fair value for interest rate sensitive instruments has been assessed on a hypothetical 10 basis point adverse movement across all maturities. As of December 31, 2024, we estimate that such hypothetical 10 basis point adverse movement would result in a hypothetical loss in fair value of approximately $0.3 million to our interest rate sensitive instruments.

Our $1,150.0 million aggregate principal amount of our 2027 Notes has a fixed interest rate of 1.25% per annum, payable semi-annually in cash on each March 15 and September 15, and therefore is not subject to fluctuations in market interest rates.

Item 8. Financial Statements and Supplementary Data.

The information required by this Item 8 begins on page F-1 in Item 15 of Part IV of this Annual Report on Form 10-K and is incorporated into this item by reference.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.

None.

Item 9A. Controls and Procedures.

Disclosure Controls and Procedures

We carried out an evaluation as of the end of the period covered by this Annual Report on Form 10-K, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of our disclosure controls and procedures pursuant to paragraph (b) of Rule 13a-15 and 15d-15 under the Exchange Act. Based on that review, the principal executive officer and principal financial officer have concluded that our disclosure controls and procedures are effective to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act (1) is recorded, processed, summarized, and reported within the time periods specified in the SEC rules and forms, and (2) is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure.

We do not expect that our disclosure controls and procedures will prevent all errors and all fraud. A control procedure, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control procedure are met. Because of the inherent limitations in all control procedures, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within our Company have been detected. These inherent limitations include the realities that judgments in decision making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by management override of the control. We considered these limitations during the development of our disclosure controls and procedures, and will continually reevaluate them to ensure they provide reasonable assurance that such controls and procedures are effective.

Internal Control over Financial Reporting

Management's Annual Report on Internal Control over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting for our Company, as such term is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act.

Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles, and includes those policies and procedures that:

- pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of our assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on our financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate. Management assessed the effectiveness of our internal control over financial reporting as of December 31, 2024. In making this assessment, management used the criteria set forth by the *Committee of Sponsoring Organizations of the Treadway Commission* in its 2013 Internal Control Integrated Framework.

Based on this assessment, management has concluded that, as of December 31, 2024, our internal control over financial reporting was effective based on those criteria.

The effectiveness of our internal control over financial reporting as of December 31, 2024, has been audited by KPMG LLP, an independent registered public accounting firm, as stated in their report which appears in this Annual Report on Form 10-K.

Changes in Internal Control over Financial Reporting

There have not been material changes in our internal control over financial reporting as defined in Rules 13a–15(f) and 15d–15(f) under the Exchange Act for the quarter ended December 31, 2024 that our certifying officers concluded materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

During the three months ended December 31, 2024, the Michael A. Chambers Living Trust, an affiliate of Michael Chambers, entered into and adopted a written plan for the sale of our securities that is intended to satisfy the conditions specified in Rule 10b5-1(c) under the Exchange Act for an affirmative defense against liability for trading in securities on the basis of material nonpublic information (a "Rule 10b5-1 trading plan"). Mr. Chambers' 10b5-1 trading plan has an adoption date of December 16, 2024 and an end date of September 12, 2025, covering 88,286 shares of common stock. As of the date of this Annual Report, Mr. Chambers beneficially owns 292,311 shares of common stock, inclusive of the shares of common stock underlying the plan.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information regarding our directors and executive officers required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2025 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

Item 11. Executive Compensation.

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2025 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2025 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2025 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

Item 14. Principal Accounting Fees and Services.

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2025 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

(a) The following documents are filed as part of this Annual Report on Form 10-K:

(1) Financial Statements

The following consolidated financial statements of the Company and the Report of KPMG LLP, Independent Registered Public Accounting Firm, are included in Part IV of this Annual Report on Form 10-K on the pages indicated:

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(2) Financial Statement Schedules

All schedules are omitted because they are not applicable or the required information is shown in the consolidated financial statements or the notes thereto.

(3) Exhibits

The exhibits required by Item 601 of Regulation S-K are listed in paragraph (b) below.

(b) Exhibits.

The following exhibits are filed herewith or are incorporated by reference to exhibits filed with the SEC:

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
2.1	Agreement and Plan of Merger dated June 6, 2013 between Sarepta Therapeutics, Inc., a Delaware corporation, and Sarepta Therapeutics, Inc., an Oregon corporation.	8-K12B	001-14895	2.1	6/6/13	
2.2*	Warrant to Purchase Common Stock of Myonexus Therapeutics, Inc., issued by Myonexus Therapeutics, Inc. to Sarepta Therapeutics, Inc., dated as of May 3, 2018.	10-Q	001-14895	2.1	8/8/18	
3.1	Amended and Restated Certificate of Incorporation.	8-K12B	001-14895	3.1	6/6/13	
3.2	Amendment to the Amended and Restated Certificate of Incorporation.	8-K	001-14895	3.1	6/30/15	
3.3	Second Amended and Restated ByLaws of Sarepta Therapeutics, Inc.	8-K	001-14895	3.1	12/13/22	
3.4	Amendment No. 1 to Second Amended and Restated Bylaws of Sarepta Therapeutics, Inc.	8-K	001-14895	3.1	9/16/24	
4.1	Form of Specimen Certificate for Common Stock.	10-Q	001-14895	4.1	8/8/13	
4.2	Indenture, dated as of September 16, 2022, by and between Sarepta Therapeutics, Inc. and U. S. Bank Trust Company, National Association (including the form of the 1.250% Convertible Senior Note due 2027).	8-K	001-14895	4.1	9/19/22	

4.3	Form of 2027 Note (included in Exhibit 4.4)	8-K	001-14895	4.2	9/19/22
4.4	Description of Registered Securities	10-K	001-14895	4.4	2/26/20
10.1†	Sarepta Therapeutics, Inc. Amended and Restated 2011 Equity Incentive Plan.	8-K	001-14895	10.1	7/1/16
10.2†	Form of Stock Option Award Agreement under the Amended and Restated 2011 Equity Incentive Plan.	10-K	001-14895	10.13	2/28/17
10.3†	Form of Restricted Stock Agreement under the Amended and Restated 2011 Equity Incentive Plan.	10-K	001-14895	10.14	2/28/17
10.4†	Form of Restricted Stock Unit Award Agreement under 2011 Equity Incentive Plan.	10-K	001-14895	10.17	2/28/17
10.5†	Form of Stock Appreciation Right Award Agreement under the 2011 Equity Incentive Plan.	10-K	001-14895	10.18	2/28/17
10.6†	Sarepta Therapeutics, Inc. Amended and Restated 2013 Employee Stock Purchase Plan.	8-K	001-14895	10.2	7/1/16
10.7†	Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan, as amended.	S-8	001-14895	4.4	2/25/16
10.8†	Form of Stock Option Award Agreement under 2014 Employment Commencement Incentive Plan	10-K	001-14895	10.28	3/3/14
10.9*	Amended and Restated Exclusive License Agreement by and among The University of Western Australia, Sarepta Therapeutics, Inc., and Sarepta International CV dated April 10, 2013.	10-Q	001-14895	10.1	5/9/13
10.10*	First Amendment to License Agreement by and among The University of Western Australia, Sarepta Therapeutics, Inc., and Sarepta International CV dated June 19, 2016.	10-Q	001-14895	10.1	8/9/16
10.11†	Amendment No. 1 to the Sarepta Therapeutics, Inc. Amended and Restated 2011 Equity Incentive Plan	8-K	001-14895	10.1	6/30/15
10.12	Asset Purchase Agreement dated February 20, 2017 by and between Sarepta Therapeutics Inc. and Gilead Sciences, Inc.	10-Q	001-14895	10.1	5/4/17
10.13†	Employment Agreement, dated as of June 26, 2017, between Sarepta Therapeutics, Inc. and Douglas S. Ingram	8-K	001-14895	10.1	6/28/17
10.14†	Change in Control and Severance Agreement by and between Douglas S. Ingram and Sarepta Therapeutics, Inc., effective June 26, 2017	8-K	001-14895	10.2	6/28/17
10.15†	Amendment No. 1 to the Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan	8-K	001-14895	10.3	6/28/17
10.16†	Restricted Stock Agreement under the 2014 Employment Commencement Incentive Plan	8-K	001-14895	10.4	6/28/17
10.17†	Performance Stock Option Award Agreement under the 2014 Employment Commencement Incentive Plan	8-K	001-14895	10.5	6/28/17
10.18*	Settlement Agreement between Sarepta Therapeutics, Inc., Sarepta International C.V. and The University of Western Australia on the one hand, and BioMarin Leiden Holding BV, BioMarin Nederlands BV and BioMarin Technologies BV on the other hand dated July 17, 2017	10-Q	001-14895	10.7	8/3/17

10.19*	License Agreement between Sarepta Therapeutics, Inc. and Sarepta International C.V. on the one hand and BioMarin Leiden Holding BV, BioMarin Nederlands BV and BioMarin Technologies BV on the other hand dated July 17, 2017	10-Q	001-14895	10.8	8/3/17
10.20	Base Call Option Transaction Confirmation, dated as of September 13, 2022, between Sarepta Therapeutics, Inc. and Barclays Bank PLC.	8-K	001-14895	10.3	9/19/22
10.21	Base Call Option Transaction Confirmation, dated as of September 13, 2022, between Sarepta Therapeutics, Inc. and Goldman Sachs & Co. LLC.	8-K	001-14895	10.4	9/19/22
10.22	Base Call Option Transaction Confirmation, dated as of September 13, 2022, between Sarepta Therapeutics, Inc. and Mizuho Markets Americas LLC.	8-K	001-14895	10.5	9/19/22
10.23	Base Call Option Transaction Confirmation, dated as of September 13, 2022, between Sarepta Therapeutics, Inc. and Morgan Stanley & Co. LLC.	8-K	001-14895	10.6	9/19/22
10.24	Base Call Option Transaction Confirmation, dated as of September 13, 2022, between Sarepta Therapeutics, Inc. and RBC Capital Markets, LLC.	8-K	001-14895	10.7	9/19/22
10.25	Additional Call Option Transaction Confirmation, dated as of September 14, 2022 between Sarepta Therapeutics, Inc. and Barclays Bank PLC.	8-K	001-14895	10.8	9/19/22
10.26	Additional Call Option Transaction Confirmation, dated as of September 14, 2022 between Sarepta Therapeutics, Inc. and Goldman Sachs & Co. LLC.	8-K	001-14895	10.9	9/19/22
10.27	Additional Call Option Transaction Confirmation, dated as of September 14, 2022 between Sarepta Therapeutics, Inc. and Mizuho Markets Americas LLC.	8-K	001-14895	10.10	9/19/22
10.28	Additional Call Option Transaction Confirmation, dated as of September 14, 2022 between Sarepta Therapeutics, Inc. and Morgan Stanley & Co. LLC.	8-K	001-14895	10.11	9/19/22
10.29	Additional Call Option Transaction Confirmation, dated as of September 14, 2022 between Sarepta Therapeutics, Inc. and RBC Capital markets, LLC.	8-K	001-14895	10.12	9/19/22
10.30†	Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan	10-Q	001-14895	10.1	8/8/18
10.31†	Letter Agreement between Douglas S. Ingram and Sarepta Therapeutics, Inc. dated June 26, 2018	10-Q	001-14895	10.4	8/8/18
10.32†	Form of Restricted Stock Unit Award Agreement under Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan	10-Q	001-14895	10.5	8/8/18
10.33†	Amendment No. 2 to the Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan	10-Q	001-14895	10.6	8/8/18
10.34†	Form of Stock Option Award Agreement under Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan	10-Q	001-14895	10.1	10/31/18

10.35†	Form of Restricted Stock Award Agreement under Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan	10-Q	001-14895	10.2	10/31/18
10.36†	Form of Restricted Stock Unit Award Agreement under Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan	10-Q	001-14895	10.3	10/31/18
10.37†	Form of Stock Appreciation Right Award Agreement under Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan	10-Q	001-14895	10.4	10/31/18
10.38†	Form of Performance-Based Restricted Stock Unit Award Agreement under Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan	10-Q	001-14895	10.1	05/4/22
10.39†	Amendment to Restricted Stock Award Agreement between Douglas S. Ingram and Sarepta Therapeutics, Inc. dated December 17, 2018	10-K	001-14895	10.75	2/28/19
10.40^	Amendment No. 1 to License Agreement between Sarepta Therapeutics, Inc. and ST International Holdings Two, Inc. on the one hand and BioMarin Leiden Holding BV, BioMarin Nederlands BV and BioMarin Technologies BV on the other hand	10-Q	001-14895	10.1	8/7/19
10.41†	Amendment No. 1 to the Sarepta Therapeutics, Inc. Amended and Restated 2013 Employment Stock Purchase Plan (as Amended and Restated on June 27, 2016)	10-Q	001-14895	10.4	8/7/19
10.42	Letter Agreement between Sarepta Therapeutics, Inc. and Myonexus Therapeutics, Inc. dated February 26, 2019	10-Q	001-14895	10.1	5/8/19
10.43†	Form of Executive Vice President Severance Letter Agreement	10-Q	001-14895	10.2	5/8/19
10.44†	Form of Executive Vice President Change in Control and Severance Agreement	10-Q	001-14895	10.3	5/8/19
10.45^	License, Collaboration, and Option Agreement between Sarepta Therapeutics Three, LLC and F. Hoffman-La Roche Ltd dated December 21, 2019	10-K	001-14895	10.51	2/26/20
10.46†	Director Compensation Policy	10-K	001-14895	10.55	2/26/20
10.47†	Amendment No. 2 to the Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan	8-K	001-14895	10.1	2/21/20
10.48†	Amendment No. 1 to the Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan	8-K	001-14895	10.1	6/8/2020
10.49†	Amendment No. 2 to the Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan	10-Q	001-14895	10.1	8/2/2022
10.50†	Promotion Letter dated December 14, 2020 by and between Sarepta Therapeutics, Inc. and Louise Rodino-Klapac	10-K	001-14895	10.59	3/1/21
10.51†	Offer Letter dated April 19, 2018 by and between Sarepta Therapeutics, Inc. and Louise Rodino-Klapac	10-K	001-14895	10.60	3/1/21
10.52†	Promotion Letter dated December 14, 2020 by and between Sarepta Therapeutics, Inc. and Ian M. Estepan	10-K	001-14895	10.61	3/1/21

10.53†	Offer Letter dated by December 18, 2014 and between Sarepta Therapeutics, Inc. and Ian M. Estepan	10-K	001-14895	10.62	3/1/21
10.54	Amendment no. 1 dated October 23, 2020 to the License, Collaboration, and Option Agreement between Sarepta Therapeutics Three, LLC and F. Hoffman-La Roche Ltd dated December 21, 2019	10-Q	001-14895	10.1	8/4/21
10.55	Amendment no. 14 dated October 31, 2022 to the License, Collaboration, and Option Agreement between Sarepta Therapeutics Three, LLC and F. Hoffman-La Roche Ltd, dated December 21, 2019	10-K	001-14895	10.74	2/28/24
10.56^	Amendment No. 2, dated November 17, 2021 to License Agreement between Sarepta Therapeutics, Inc. and ST International Holdings Two, Inc. on the one hand and BioMarin Leiden Holding BV, BioMarin Nederlands BV and BioMarin Technologies BV on the other hand	10-K	001-14895	10.66	3/1/22
10.57†	Letter Agreement, dated November 18, 2022, between Sarepta Therapeutics, Inc. and Douglas S. Ingram	10-K	001-14895	10.72	2/28/23
10.58	Sarepta Therapeutics Inc.'s Policy for Recoupment of Incentive Compensation	10-K	001-14895	10.77	2/28/24
10.59^	Amended and Restated Lead DMD Product Manufacturing and Supply Agreement between Catalent Maryland, Inc. and Sarepta Therapeutics Three, LLC	10-Q	001-14895	10.1	8/2/23
10.60^	Exclusive License Agreement between the Research Institute at Nationwide Children's Hospital and Sarepta Therapeutics, Inc., dated October 8, 2018	10-Q	001-14895	10.2	8/2/23
10.61^	First Amendment to the Amended and Restated Lead DMD Product Manufacturing & Supply Agreement between Catalent Maryland, Inc. and Sarepta Therapeutics Three, LLC	10-Q	001-14895	10.5	8/2/23
10.62	Amendment No. 2 to the Sarepta Therapeutics, Inc. Amended and Restated 2013 Employee Stock Purchase Plan	8-K	001-14895	10.2	6/9/23
10.63	Amendment No. 3 to the Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan	8-K	001-14895	10.1	6/9/23
10.64^	First Amendment, dated May 29, 2019, to the Exclusive License Agreement between Research Institute at Nationwide Children's Hospital and Sarepta Therapeutics, Inc.	10-Q	001-14895	10.3	8/2/23
10.65^	Second Amendment, dated July 11, 2023, to the Exclusive License Agreement between Research Institute at Nationwide Children's Hospital and Sarepta Therapeutics, Inc.	10-Q	001-14895	10.4	8/2/23
10.66	Credit Agreement, dated February 13, 2025, among Sarepta Therapeutics, Inc., Sarepta Therapeutics	8-K	001-14895	10.1	2/14/25

	Investments, Inc., JPMorgan Chase Bank, N.A., as administrative agent, and the lenders party thereto	
10.67^	Exclusive License and Collaboration Agreement, dated November 25, 2024, between Sarepta Therapeutics, Inc. and Arrowhead Pharmaceuticals, Inc.	X
19.1	Sarepta Therapeutics, Inc. Insider Trading Policy	X
21.1	Subsidiaries of the Registrant.	X
23.1	Consent of Independent Registered Public Accounting Firm.	X
24.1	Power of Attorney (contained on signature page).	X
31.1	Certification of the Company's President and Chief Executive Officer, Douglas S. Ingram, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.	X
31.2	Certification of the Company's Executive Vice President, Chief Financial Officer, Ian Estepan, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.	X
32.1**	Certification of the Company's President and Chief Executive Officer, Douglas S. Ingram, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.	X
32.2**	Certification of the Company's Executive Vice President, Chief Financial Officer, Ian Estepan, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.	X
101.INS	Inline XBRL Instance Document - the instance document does not appear in the Interactive Data File as its XBRL tags are embedded within the Inline XBRL document.	X
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents	X
104	The Cover Page from the Annual Report on Form 10-K of Sarepta Therapeutics, Inc. for the year ended December 31, 2024, formatted in Inline XBRL	X

† Indicates management contract or compensatory plan, contract or arrangement.
^ Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.
* Confidential treatment has been granted for portions of this exhibit.
** Furnished herewith. This exhibit shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, or otherwise subject to the liability of that Section. Such exhibit shall not be deemed incorporated into any filing under the Securities Act of 1933 or the Securities Exchange Act of 1934.

Item 16. Form 10-K Summary.

Not applicable.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Dated: February 28, 2025

SAREPTA THERAPEUTICS, INC.

By: /s/ Douglas S. Ingram
Douglas S. Ingram
President and Chief Executive Officer

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Douglas S. Ingram and Ian M. Estepan, and each of them, with full power of substitution and resubstitution and full power to act without the other, as his or her true and lawful attorney-in-fact and agent to act in his or her name, place and stead and to execute in the name and on behalf of each person, individually and in each capacity stated below, and to file, any and all documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing, ratifying and confirming all that said attorneys-in-fact and agents or any of them or their and his or her substitute or substitutes, may lawfully do or cause to be done by virtue thereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities indicated on February 28, 2025:

Signature	Title
/s/ Douglas S. Ingram Douglas S. Ingram	President, Chief Executive Officer and Director (Principal Executive Officer)
/s/ Ian M. Estepan Ian M. Estepan	Executive Vice President, Chief Financial Officer (Principal Financial and Accounting Officer)
/s/ M. Kathleen Behrens M. Kathleen Behrens, Ph.D.	Chairwoman of the Board
/s/ Richard Barry Richard Barry	Director
/s/ Deirdre Connelly Deirdre Connelly	Director
/s/ Kathryn Boor Kathryn J. Boor, Ph.D.	Director
/s/ Michael A. Chambers Michael A. Chambers	Director
/s/ Stephen L. Mayo Stephen L. Mayo, Ph.D.	Director
/s/ Claude Nicaise Claude Nicaise, MD	Director
/s/ Hans Wigzell Hans Wigzell, M.D., Ph.D.	Director

SAREPTA THERAPEUTICS, INC.
CONSOLIDATED FINANCIAL STATEMENTS

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Report of Independent Registered Public Accounting Firm

To the Stockholders and Board of Directors
Sarepta Therapeutics, Inc.:

Opinions on the Consolidated Financial Statements and Internal Control Over Financial Reporting

We have audited the accompanying consolidated balance sheets of Sarepta Therapeutics, Inc. and subsidiaries (the Company) as of December 31, 2024 and 2023, the related consolidated statements of comprehensive income (loss), stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2024, and the related notes (collectively, the consolidated financial statements). We also have audited the Company's internal control over financial reporting as of December 31, 2024, based on criteria established in *Internal Control – Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of the Company as of December 31, 2024 and 2023, and the results of its operations and its cash flows for each of the years in the three-year period ended December 31, 2024, in conformity with U.S. generally accepted accounting principles. Also in our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2024 based on criteria established in *Internal Control – Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission.

Basis for Opinions

The Company's management is responsible for these consolidated financial statements, for maintaining effective internal control over financial reporting, and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Annual Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the Company's consolidated financial statements and an opinion on the Company's internal control over financial reporting based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud, and whether effective internal control over financial reporting was maintained in all material respects.

Our audits of the consolidated financial statements included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audits also included performing such other procedures as we considered necessary in the circumstances. We believe that our audits provide a reasonable basis for our opinions.

Definition and Limitations of Internal Control Over Financial Reporting

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the consolidated financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the consolidated financial statements and (2) involved our especially challenging, subjective, or complex judgments. The communication of a critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

Evaluation of lower of cost or net realizable value of raw materials inventory

As described in Note 8 to the consolidated financial statements, approximately 30%, or $280.0 million, of the Company's total inventory balance is comprised of raw materials. As discussed in Note 2, the Company periodically analyzes its inventories, and writes down obsolete or otherwise unmarketable inventory to its estimated net realizable value.

We identified the evaluation of lower of cost or net realizable value of certain raw materials inventory as a critical audit matter. The estimate of expected future demand for raw materials inventory is difficult to assess and results in the application of greater auditor judgment. Specifically, challenging auditor judgment was required to assess the potential impact the Company's gene therapy technologies and competitor RNA-targeted therapeutic or gene therapy products could have on certain existing raw materials inventory.

The following are the primary procedures we performed to address this critical audit matter. We evaluated the design and tested the operating effectiveness of a control related to the future demand for raw materials. We compared the Company's prior period forecasted demand for raw materials to actual results to assess their ability to accurately estimate expected future demand. We evaluated clinical progress associated with the Company's gene therapy technologies, including the limited approval by the FDA of ELEVIDYS during 2023 and expanded approval in 2024, by inspecting internal meeting minutes and interviewing research and development personnel of the Company and assessed the potential impact of those technologies on expected future demand for certain raw materials inventory. We inquired of manufacturing personnel and evaluated the expiry of certain raw materials. We also read publicly available information to identify other competitor entities with RNA-targeted therapeutic or gene therapy products that could impact the Company's estimates of expected future demand.

/s/ KPMG LLP

We have served as the Company's auditor since 2002.

Boston, Massachusetts

February 28, 2025

Sarepta Therapeutics, Inc.

Consolidated Balance Sheets

(in thousands, except share and per share amounts)

	As of December 31,	
	2024	2023
Assets		
Current assets:		
Cash and cash equivalents	$ 1,103,010	$ 428,430
Short-term investments	251,782	1,247,820
Accounts receivable, net	601,988	400,327
Inventory	749,960	322,859
Manufacturing-related deposits and prepaids	276,262	102,181
Other current assets	90,461	77,714
Total current assets	3,073,463	2,579,331
Property and equipment, net	340,336	227,154
Right of use assets	148,310	129,952
Non-current inventory	187,986	191,368
Non-current investments	133,163	—
Other non-current assets	79,915	136,771
Total assets	$ 3,963,173	$ 3,264,576
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 214,442	$ 164,918
Accrued expenses	373,513	314,997
Deferred revenue, current portion	130,256	50,416
Current portion of long-term debt	—	105,483
Other current liabilities	13,473	17,845
Total current liabilities	731,684	653,659
Long-term debt	1,137,124	1,132,515
Lease liabilities, net of current portion	192,473	140,965
Deferred revenue, net of current portion	325,000	437,000
Contingent consideration	47,400	38,100
Other non-current liabilities	1,750	3,000
Total liabilities	2,435,431	2,405,239
Commitments and contingencies (Note 22)		
Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 198,000,000 shares authorized; 96,900,496 and 93,731,831 issued and outstanding at December 31, 2024 and 2023, respectively	10	9
Additional paid-in capital	5,738,924	5,304,623
Accumulated other comprehensive (loss) income, net of tax	(218)	918
Accumulated deficit	(4,210,974)	(4,446,213)
Total stockholders' equity	1,527,742	859,337
Total liabilities and stockholders' equity	$ 3,963,173	$ 3,264,576

See accompanying notes to consolidated financial statements.

Sarepta Therapeutics, Inc.

Consolidated Statements of Comprehensive Income (Loss)

(in thousands, except per share data)

	For the Year Ended December 31,		
	2024	**2023**	**2022**
Revenues:			
Products, net	$ 1,787,960	$ 1,144,876	$ 843,769
Collaboration and other	114,019	98,460	89,244
Total revenues	1,901,979	1,243,336	933,013
Cost and expenses:			
Cost of sales (excluding amortization of in-licensed rights)	319,099	150,343	139,989
Research and development	804,522	877,387	877,090
Selling, general and administrative	557,872	481,871	451,421
Amortization of in-licensed rights	2,405	1,559	714
Total cost and expenses	1,683,898	1,511,160	1,469,214
Operating income (loss)	218,081	(267,824)	(536,201)
Other income (loss), net:			
Other income (expense), net	42,693	33,055	(28,321)
Loss on debt extinguishment	—	(387,329)	(125,441)
Gain from sale of Priority Review Voucher	—	102,000	—
Total other income (loss), net	42,693	(252,274)	(153,762)
Income (loss) before income tax expense	260,774	(520,098)	(689,963)
Income tax expense	25,535	15,879	13,525
Net income (loss)	$ 235,239	$ (535,977)	$ (703,488)
Other comprehensive income (loss):			
Unrealized (losses) gains on investments, net of tax	$ (1,136)	$ 2,582	$ (1,644)
Total other comprehensive (loss) income	(1,136)	2,582	(1,644)
Comprehensive income (loss)	$ 234,103	$ (533,395)	$ (705,132)
Earnings (loss) per share:			
Basic	$ 2.47	$ (5.80)	$ (8.03)
Diluted	$ 2.34	$ (5.80)	$ (8.03)
Weighted average number of shares of common stock used in computing earnings (loss) per share:			
Basic	95,075	92,398	87,559
Diluted	107,875	92,398	87,559

See accompanying notes to consolidated financial statements.

Sarepta Therapeutics, Inc.

Consolidated Statements of Stockholders' Equity

(in thousands)

	Common Stock Shares	Common Stock Amount	Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Total Stockholders' Equity
BALANCE AT DECEMBER 31, 2021	**87,127**	**$ 9**	**$ 4,134,768**	**$ (20)**	**$ (3,206,748)**	**$ 928,009**
Exercise of options for common stock	318	—	22,573	—	—	22,573
Vest of restricted stock units	389	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	116	—	7,470	—	—	7,470
Stock-based compensation	—	—	233,018	—	—	233,018
Purchase of capped call share options for 2027 Notes	—	—	(127,305)	—	—	(127,305)
Partial settlement of capped call share options for 2024 Notes	—	—	26,317	—	—	26,317
Unrealized losses from available-for-sale securities, net of tax	—	—	—	(1,644)	—	(1,644)
Net loss	—	—	—	—	(703,488)	(703,488)
BALANCE AT DECEMBER 31, 2022	**87,950**	**9**	**4,296,841**	**(1,664)**	**(3,910,236)**	**384,950**
Exercise of options for common stock	528	—	40,485	—	—	40,485
Vest of restricted stock units	645	—	—	—	—	—
Issuance of common stock for exchange of 2024 Notes	4,456	—	693,377	—	—	693,377
Partial settlement of capped call share options for 2024 Notes	—	—	80,645	—	—	80,645
Issuance of common stock under employee stock purchase plan	153	—	10,761	—	—	10,761
Stock-based compensation	—	—	182,514	—	—	182,514
Unrealized gains from available-for-sale securities, net of tax	—	—	—	2,582	—	2,582
Net loss	—	—	—	—	(535,977)	(535,977)
BALANCE AT DECEMBER 31, 2023	**93,732**	**9**	**5,304,623**	**918**	**(4,446,213)**	**859,337**
Exercise of options for common stock	850	1	67,319	—	—	67,320
Vest of restricted stock units	734	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	143	—	12,205	—	—	12,205
Issuance of common stock for conversion of 2024 Notes	1,441	—	105,757	—	—	105,757
Modification of 2017 Capped Calls	—	—	43,887	—	—	43,887
Stock-based compensation	—	—	205,133	—	—	205,133
Unrealized losses from available-for-sale securities, net of tax	—	—	—	(1,136)	—	(1,136)
Net income	—	—	—	—	235,239	235,239
BALANCE AT DECEMBER 31, 2024	**96,900**	**$ 10**	**$ 5,738,924**	**$ (218)**	**$ (4,210,974)**	**$ 1,527,742**

See accompanying notes to consolidated financial statements.

Sarepta Therapeutics, Inc.
Consolidated Statements of Cash Flows
(in thousands)

	For the Year Ended December 31,		
	2024	**2023**	**2022**
Cash flows from operating activities:			
Net income (loss)	$ 235,239	$ (535,977)	$ (703,488)
Depreciation and amortization	37,724	44,397	41,864
Reduction in the carrying amounts of the right of use assets	16,167	14,495	12,735
Non-cash interest expense	4,951	5,156	7,552
Stock-based compensation	184,300	182,514	233,018
Accretion of investment discount, net	(40,277)	(46,176)	(10,651)
Non-cash termination charges	62,747	—	—
Non-cash change in the fair value of derivatives	7,838	1,200	(6,700)
Impairment of strategic investments	—	30,321	2,575
Loss on debt extinguishment	—	387,329	125,441
Gain from sale of Priority Review Voucher	—	(102,000)	—
Other	2,130	(1,163)	11,778
Changes in operating assets and liabilities, net:			
Increase in accounts receivable	(201,661)	(185,699)	(61,638)
(Increase) decrease in manufacturing-related deposits and prepaids	(188,588)	(12,521)	42,557
Increase in inventory	(395,170)	(147,714)	(50,780)
(Increase) decrease in other assets	(9,676)	1,808	(27,937)
Decrease in deferred revenue	(32,160)	(86,828)	(89,244)
Increase (decrease) in accounts payable, accrued expenses, lease liabilities and other liabilities	110,649	(50,135)	147,572
Net cash used in operating activities	(205,787)	(500,993)	(325,346)
Cash flows from investing activities:			
Purchase of property and equipment	(136,956)	(76,106)	(30,824)
Purchase of available-for-sale securities	(1,099,595)	(2,044,940)	(1,936,856)
Maturity and sales of available-for-sale securities	2,002,112	1,868,482	923,224
Purchase of intangible assets	(10,000)	(11,239)	(1,427)
Acquisition of strategic investments	—	(4,000)	(1,000)
Proceeds from sale of Priority Review Voucher	—	102,000	—
Net cash provided by (used in) investing activities	755,561	(165,803)	(1,046,883)
Cash flows from financing activities:			
Settlement of capped call share options for 2024 Notes	45,349	80,645	26,317
Proceeds from exercise of stock options and purchase of stock under the Employee Stock Purchase Program	79,525	51,246	30,043
Payment on maturity of 2024 Notes	(68)	—	—
Debt conversion costs for 2024 Notes	—	(6,887)	—
Proceeds from 2027 Notes offering, net of commissions	—	—	1,127,400
Purchase of capped call share options for 2027 Notes	—	—	(127,305)
Debt issuance costs for 2027 Notes	—	—	(716)
Repurchase of 2024 Notes	—	—	(247,868)
Repayment of principal amount due under 2019 Term Loan	—	—	(550,000)
Payment on debt extinguishment of 2019 Term Loan	—	—	(25,364)
Net cash provided by financing activities	124,806	125,004	232,507
Increase (decrease) in cash and cash equivalents	674,580	(541,792)	(1,139,722)
Cash, cash equivalents and restricted cash:			
Beginning of year	444,009	985,801	2,125,523
End of year	$ 1,118,589	$ 444,009	$ 985,801
Reconciliation of cash, cash equivalents and restricted cash:			
Cash and cash equivalents	$ 1,103,010	$ 428,430	$ 966,777
Restricted cash in other assets	15,579	15,579	19,024
Total cash, cash equivalents and restricted cash	$ 1,118,589	$ 444,009	$ 985,801
Supplemental disclosure of cash flow information:			
Cash paid during the period for interest	$ 15,856	$ 15,923	$ 44,418
Cash paid during the period for income taxes	$ 22,587	$ 15,081	$ 1,695
Supplemental schedule of non-cash activities:			
Common stock issued for conversion or exchange of 2024 Notes	$ 105,757	$ 693,377	$ —
Intangible assets and property and equipment included in accounts payable and accrued expenses	$ 42,740	$ 33,339	$ 6,765
Lease liabilities arising from obtaining right of use assets	$ 35,361	$ 80,203	$ 40,006
Capitalized stock-based compensation and depreciation as inventory	$ 28,549	$ —	$ —
Lease liabilities terminated	$ 2,381	$ —	$ 3,807

See accompanying notes to consolidated financial statements.

1. ORGANIZATION AND NATURE OF BUSINESS

Sarepta Therapeutics, Inc. (together with its wholly-owned subsidiaries, "Sarepta" or the "Company") is a commercial-stage biopharmaceutical company focused on helping patients through the discovery and development of unique RNA-targeted therapeutics, gene therapy and other genetic therapeutic modalities for the treatment of rare diseases. Applying its proprietary, highly-differentiated and innovative technologies, and through collaborations with its strategic partners, the Company has developed multiple approved products for the treatment of Duchenne muscular dystrophy ("Duchenne") and is developing potential therapeutic candidates for a broad range of diseases and disorders, including Duchenne, Limb-girdle muscular dystrophies ("LGMDs") and other neuromuscular and central nervous system ("CNS") disorders.

The Company's products in the U.S., EXONDYS 51 (eteplirsen) Injection ("EXONDYS 51"), VYONDYS 53 (golodirsen) Injection ("VYONDYS 53") and AMONDYS 45 (casimersen) Injection ("AMONDYS 45"), were granted accelerated approval by the U.S. Food and Drug Administration (the "FDA") in 2016, 2019 and 2021, respectively. Indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 51, exon 53 and exon 45 skipping, respectively, EXONDYS 51, VYONDYS 53 and AMONDYS 45 (collectively, the "PMO Products") use the Company's phosphorodiamidate morpholino oligomer ("PMO") chemistry and exon-skipping technology to skip exon 51, exon 53 and exon 45 of the dystrophin gene. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein.

ELEVIDYS (delandistrogene moxeparvovec-rokl), approved by the FDA on June 20, 2024, is an adeno-associated virus-("AAV") based gene therapy for the treatment of ambulatory patients at least four years old with Duchenne with a confirmed mutation in the Duchenne gene. ELEVIDYS is also approved for non-ambulatory patients under the accelerated approval pathway. ELEVIDYS was previously granted accelerated approval by the FDA in June 2023 for the treatment of ambulatory patients aged four through five years with Duchenne with a confirmed mutation in the Duchenne gene. ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the Duchenne gene.

As of December 31, 2024, the Company had approximately $1,503.5 million of cash, cash equivalents, restricted cash and investments, consisting of $1,103.0 million of cash and cash equivalents, $384.9 million of investments and $15.6 million of restricted cash. The Company believes that its balance of cash, cash equivalents and investments as of the date of the issuance of this report is sufficient to fund its current operational plan for at least the next twelve months, though it may pursue additional cash resources through public or private debt and equity financings, seek funded research and development arrangements and additional government contracts and establish collaborations with or license its technology to other companies.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES AND RECENT ACCOUNTING PRONOUNCEMENTS

Basis of Presentation

The accompanying consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States ("U.S. GAAP"), reflect the accounts of Sarepta and its wholly-owned subsidiaries. All intercompany transactions between and among its consolidated subsidiaries have been eliminated. All adjustments of a normal recurring nature necessary for a fair presentation have been reflected.

Segments

Management has determined that the Company operates in one segment: discovering, developing, manufacturing and delivering therapies to patients with rare diseases. The Company's Chief Executive Officer ("CEO"), as the chief operating decision-maker ("CODM"), manages and allocates resources to the operations of the Company on a total company basis. For more information related to the Company's segment results, please read *Note 21, Segment Information*.

Estimates and Uncertainties

The preparation of the consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, equity, revenue, expenses and the disclosure of contingent assets and liabilities. Actual results could differ from those estimates.

Fair Value Measurements

The Company has certain financial assets and liabilities that are recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements:

- Level 1—quoted prices for identical instruments in active markets;
- Level 2—quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active, and model-derived valuations in which all significant inputs and significant value drivers are observable in active markets; and
- Level 3—valuations derived from valuation techniques in which one or more significant value drivers are unobservable.

The fair value of the majority of the Company's financial assets is categorized as Level 2 within the fair value hierarchy. These assets include commercial paper, government and government agency bonds, corporate bonds and certificates of deposit. For additional information related to fair value measurements, please read *Note 5, Fair Value Measurements* to the consolidated financial statements.

Cash Equivalents

Only investments that are highly liquid and readily convertible to cash and have original maturities of three months or less at the time of acquisition are considered cash equivalents.

Investments

Available-For-Sale Debt Securities

Available-for-sale debt securities are recorded at fair value and unrealized gains and losses are included in accumulated other comprehensive income (loss) in the consolidated statements of stockholders' equity. Interest income and realized gains and losses are reported in other income (expense), net, on a specific identification basis. The amortized cost of debt securities is adjusted for amortization of premiums and accretion of discounts to maturity, the net amount of which, along with interest and realized gains and losses, is included in other income (expense), net in the consolidated statements of comprehensive income (loss).

Equity Investments

The Company's equity investments include its strategic investments in both publicly traded and private biotechnology companies and are included in other non-current assets in the Company's consolidated balance sheets. The strategic investment in the publicly traded biotechnology company has a readily determinable fair value and is carried at fair value. The strategic investment in the privately held biotechnology company does not have a readily determinable fair value and is measured at cost less any impairment, plus or minus changes resulting from observable price changes for the identical or a similar investment of the same issuer. Any change in the valuation of equity investments is recorded as a gain or loss on the Company's consolidated statements of comprehensive income (loss).

Accounts Receivable, Net

The Company's accounts receivable, net arise from product sales. They are generally stated at the invoiced amount and do not bear interest.

The accounts receivable, net from product sales represents receivables due from the Company's specialty distributor and specialty pharmacies and sites of care in the U.S., as well as certain distributors in South America, Europe, and the Middle East. Historically, the Company has had no material write-offs of its accounts receivable, net. Payment terms range from 60 to 90 days for sales within the U.S. and 60 and 150 days for the majority of product sales outside the U.S. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profiles or any specific issues. The Company provides reserves against trade receivables for expected credit losses that may result from a customer's inability to pay. Amounts determined to be uncollectible are written-off against the established reserve. As of December 31, 2024 and 2023, the credit profiles for the Company's customers are deemed to be in good standing and an allowance for expected credit losses is not considered necessary.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to concentrations of credit risk consist of cash held at financial institutions, cash equivalents, investments and accounts receivable, net from customers. As of December 31, 2024, the Company's cash was concentrated at three financial institutions, which potentially exposes the Company to credit risks. However, the Company does not believe that there is significant risk of non-performance by the financial institutions. The Company also purchases commercial paper, government and government agency bonds, corporate bonds and certificates of deposit issued by highly rated corporations, financial institutions and governments and limits the amount of credit exposure to any one issuer. These amounts may at times exceed federally insured limits. The Company has not experienced any credit losses related to these financial instruments and does not believe to be exposed to any significant credit risk related to these instruments. As of December 31, 2024, three entities accounted for 54%, 21%, and 13% of accounts receivable, net, respectively. As of December 31, 2023, four entities accounted for 40%, 19%, 19%, and 12% of accounts receivable, net, respectively.

Inventories

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis. The Company capitalizes inventory costs associated with products following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. EXONDYS 51, VYONDYS 53, AMONDYS 45 and ELEVIDYS inventory used in clinical development programs is charged to research and development expense when the product enters the research and development process and can no longer be used for commercial purposes.

The Company periodically analyzes its inventories for excess amounts or obsolescence and writes down obsolete or otherwise unmarketable inventory to its estimated net realizable value. Additionally, though the Company's products are subject to strict quality control and monitoring the Company performs throughout the manufacturing processes, certain batches or units of product may not meet quality specifications. Expense incurred related to excess inventory, obsolete inventory, or inventories that do not meet the Company's quality specifications is recorded as a component of cost of sales in the Company's consolidated statements of comprehensive income (loss).

For products which are under development and have not yet been approved by regulatory authorities, purchased drug product is charged to research and development expense upon delivery. Delivery occurs when the inventory passes quality inspection and ownership transfers to the Company. Nonrefundable advance payments for research and development activities, including production of purchased drug product, are deferred and capitalized until the goods are delivered. If the Company does not expect the goods to be delivered or services to be rendered, the capitalized advanced payment will be charged to expense.

Property and Equipment

Property and equipment are initially recorded at cost, including the acquisition cost and all costs necessarily incurred to bring the asset to the location and working condition necessary for their intended use. The cost of normal, recurring or periodic repairs and maintenance activities related to property and equipment are expensed as incurred. The cost for planned major maintenance activities, including the related acquisition or construction of assets, is capitalized if the repair will result in future economic benefits. Interest costs incurred during the construction period of major capital projects are periodically reviewed, and if determined to be material, capitalized until the asset is ready for its intended use, at which point the interest costs are amortized as depreciation expense over the life of the underlying asset.

The Company generally depreciates the cost of its property and equipment using the straight-line method over the estimated useful lives of the respective assets, which are summarized as follows:

Asset Category	Useful lives
Lab and manufacturing equipment	5 years
Office equipment	5 years
Software and computer equipment	3 - 5 years
Furniture and fixtures	7 years
Leasehold improvements	Lesser of the useful life or the term of the respective lease
Land improvements	25 years
Land	Not depreciated
Building and improvements	30 years
Construction in progress	Not depreciated until put into service

Intangible assets

The Company's intangible assets, consisting of in-licensed rights, patent costs and software licenses, are included within other non-current assets in the Company's consolidated balance sheets.

The in-licensed rights primarily relate to agreements with BioMarin Pharmaceutical, Inc. ("BioMarin"), the University of Western Australia ("UWA"), the Research Institute at Nationwide Children's Hospital ("Nationwide") and Parent Project Muscular Dystrophy ("PPMD"). The in-licensed rights are being amortized on a straight-line basis over the remaining life of the related patents because the life of the related patents reflects the expected time period that the Company will benefit from the in-licensed rights.

Impairment of Long-Lived Assets

Long-lived assets held and used by the Company, intangible assets with definite lives and right of use ("ROU") assets are reviewed for impairment whenever events or circumstances indicate that the carrying amount of the assets may not be recoverable. The Company evaluates recoverability of assets to be held and used by comparing the carrying amount of an asset to future net undiscounted cash flows to be generated by the asset. If the asset is considered to be impaired, the impairment to be recognized is measured as the amount by which the carrying amount of the assets exceeds the fair value of the assets. Such reviews assess the fair value of the assets based upon estimates of future cash flows that the assets are expected to generate.

Convertible Debt

The Company accounts for the liability and equity components of convertible debt instruments that can be settled in cash as a single liability measured at its amortized cost, as long as no other features require bifurcation and recognition as derivatives under ASC Topic 815, *Derivatives and Hedging* ("ASC 815"). Simultaneously with the issuance of the Company's convertible senior notes due on November 15, 2024 (the "2024 Notes") and convertible senior notes due on September 15, 2027 (the "2027 Notes") in November 2017 and September 2022, respectively, the Company bought capped call options from certain counterparties to minimize the impact of potential dilution upon conversion. The premium for the capped call options was recorded as additional paid-in capital. For additional information related to the convertible debt transactions, please read *Note 13, Indebtedness* to the consolidated financial statements.

Revenue Recognition

The Company recognizes revenue when a customer obtains control of promised goods or services, in an amount that reflects the consideration which the Company expects to receive in exchange for the goods or services provided. To determine revenue recognition for arrangements within the scope of ASC Topic 606, *Revenue from Contracts with Customers* ("ASC 606"), the Company performs the following five steps: (1) identify the contract with the customer; (2) identify the performance obligations in the contract; (3) determine the transaction price; (4) allocate the transaction price to the performance obligations in the contract; and (5) recognize revenue when or as the Company satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers or provides to the customer. At contract inception, the Company assesses the goods or services promised within each contract and determines those that are performance obligations, and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when or as the performance obligation is satisfied. The only performance obligation in the Company's contracts with customers is to timely deliver drug products to the customer's designated location.

Product revenues

The Company distributes its products principally through its customers or sells directly to sites of care. When the product is distributed through customers, the customers subsequently resell the products to patients and health care providers. The Company provides right of return to the customers only in cases of shipping error or product defect and other limited rights. Product revenues are recognized when the customers take control of the products, which typically occurs upon delivery or shipment. For information related to revenues by product type and region, please read *Note 7, Product Revenues, Net, Accounts Receivable, Net and Reserves for Product Revenues* to the consolidated financial statements.

Variable Consideration

Product revenues are recorded at the net sales price (transaction price) which includes reserves for variable consideration such as: rebates and chargebacks, distribution fees, prompt pay discounts, patient assistance and return reserves. These reserves, representing the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the contracts, are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable if no payments are required of the Company or a current liability if a payment is required of the Company. Where appropriate, the estimates reflect the Company's historical experience, contractual and statutory requirements, industry data and forecasted customer buying and payment patterns. Actual amounts may differ from the Company's estimates. If actual results vary, these estimates are adjusted, which could have an effect on revenue in the period of adjustment.

Additional details relating to variable consideration are as follows:

- Rebates and chargebacks: relating to governmental and commercial rebates and chargebacks.
    - Governmental rebates, including Medicaid-rebates relate to the Company's estimated obligations to federal or states under established reimbursement arrangements. The commercial rebates relate to arrangements the Company enters into with payors that provide for privately-negotiated rebates. Rebate reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a liability which is included in accrued expenses.
    - Chargebacks, including Public Health Services ("PHS") chargebacks, relate to the Company's estimated obligations resulting from contractual commitments to sell products to qualified healthcare providers at prices lower than the list prices that the Company charges to wholesalers. The wholesaler charges the Company for the difference between what the wholesaler pays for the products and the ultimate selling price to the qualified healthcare providers. Chargeback reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable. Chargeback amounts are generally determined at the time of resale to the qualified healthcare provider from the wholesaler, and the Company generally issues credits for such amounts within a few weeks of receiving notification of resale from the wholesaler.
- Distribution fees: relating to fees paid to customers in the distribution channel that provide the Company with inventory management, data and distribution services and are generally accounted for as a reduction of revenue. To the extent that the services received are distinct from the Company's sale of products to the customers, these payments are accounted for as selling, general and administrative expenses. Reserves for distribution fees result in an increase in a liability if payments are required of the Company or a reduction of accounts receivable if no payments are required of the Company.
- Prompt payment discounts: relating to the Company's estimated obligations for credits to be granted to specialty pharmacies for remitting payment on their purchases within established incentive periods. Reserves for prompt payment discounts are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable.
- Patient assistance: relating to financial assistance programs provided to qualified patients. Reserves for costs related to patient assistance programs are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a liability which is included in accrued expenses.
- Return reserves: relating to the limited return rights the Company provides to customers. The Company records product return reserve, if appropriate, as a reduction of revenue at the time product sales are recorded. Several factors are considered in determining whether return reserves are required, including the patient population and the customers' limited return rights. Because of the pricing, the limited number of patients, and the customers' limited return rights, most customers only carry a limited inventory. Based on these factors and the fact that the Company has not experienced significant product returns to date, return reserves have been immaterial to date.

Collaboration revenue

The Company's collaboration revenue is primarily generated from its collaboration arrangement with F. Hoffman-La Roche Ltd. ("Roche"). For more information, please read *Note 3, License and Collaboration Agreements*. At the inception of a collaboration arrangement, the Company first assesses whether the contractual arrangement is within the scope of ASC Topic 808, *Collaborative Arrangements* to determine whether the arrangement involves a joint operating activity and involves two (or more) parties that are both active participants in the activity and exposed to significant risks and rewards dependent on the commercial success of such activity. Then the Company determines whether the collaboration arrangement in its entirety represents a contract with a customer as defined by ASC 606. If only a portion of the collaboration arrangement is potentially with a customer, the Company applies the

distinct good or service unit-of-account guidance in ASC 606 to determine whether there is a unit of account that should be accounted for under ASC 606. For the units of account in the collaboration arrangement that do not represent a vendor-customer relationship, the Company will (i) consider applying other GAAP, including by analogy, or (ii) if there is no appropriate analogy, consistently apply a reasonable and rational accounting policy election.

In general, by analogy to ASC 606, the Company identifies the performance obligations within the collaboration arrangement and identifies and allocates the transaction price the Company expects to receive on a relative standalone selling price basis to each performance obligation. Variable consideration, consisting of development and regulatory milestones, will be included in the transaction price only if the Company expects to receive such consideration and if it is probable that the inclusion of the variable consideration will not result in a significant reversal in the cumulative amount of revenue recognized under the arrangement. Sales-based royalty and milestone payments are excluded from the transaction price the Company expects to receive until the underlying sales occur because the license to the Company's intellectual property is deemed to be the predominant item to which the royalties or milestones relate as it is the primary driver of value in its collaboration arrangement.

For the recognition of revenue associated with each performance obligation, if the Company determines ASC 606 is not appropriate to apply by analogy, the Company will apply a reasonable, rational and consistently applied accounting policy election to faithfully depict the transfer of services to the collaboration partner over the estimated performance period. Up-front payments from a collaboration partner are recognized as deferred revenue when received and recognized as revenue over the estimated performance period. Reimbursement payments from a collaboration partner associated with cost-sharing provisions in a collaboration arrangement are recognized as the related expense is incurred and classified as an offset to operating expenses. Revenue from sales-based royalty payments is included as collaboration and other revenues on the consolidated statements of comprehensive income (loss). Revenue from product supply sold to collaboration partners under a collaboration arrangement via contract manufacturing is included as collaboration and other revenues on the consolidated statements of comprehensive income (loss).

Valuation of Product Options

The Company's collaboration arrangements may contain options which provide the collaboration partner with the right to obtain additional licenses. If an arrangement contains product options, by analogy to ASC 606, the Company evaluates the product options to determine whether they represent material rights, which may include options to acquire additional goods or services for free or at a discount. If the customer options are determined to represent material rights, they are recognized as a separate performance obligation at inception of the arrangement. The Company allocates a portion of the transaction price of the collaboration arrangement to material rights based on the relative standalone selling price. Amounts allocated to material rights are not recognized as revenue until related options are exercised or expire. Key assumptions to determine the standalone selling price of product options in a collaboration arrangement include, but are not limited to, forecasted revenues, development timelines, incremental costs related to the arrangement, discount rates and likelihood of technical and regulatory success.

Research and Development Expenses

Research and development expenses consist of costs associated with research activities as well as those with the Company's product development efforts, conducting pre-clinical trials, clinical trials and manufacturing activities. Research and development expenses are expensed as incurred. Up-front fees and milestones paid to third parties in connection with technologies which have not reached technological feasibility and do not have an alternative future use are expensed when incurred.

Direct research and development expenses associated with the Company's programs include clinical trial site costs, clinical manufacturing costs, costs incurred for consultants and other external services, such as data management and statistical analysis support and materials and supplies used in support of clinical programs. Indirect costs of the Company's research and development programs include salaries, stock-based compensation and an allocation of its facility and technology costs.

When third-party service providers' billing terms do not coincide with the Company's period-end, the Company is required to make estimates of its obligations to those third parties, including clinical trial and pharmaceutical development costs, contractual services costs and costs for supply of its drug candidates incurred in a given accounting period, and record accruals at the end of the period. The Company bases its estimates on its knowledge of the research and development programs, services performed for the period, past history for related activities and the expected duration of the third-party service contract, where applicable.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist of salaries, benefits, stock-based compensation and related costs for personnel in the Company's executive, finance, legal, information technology, business development, human resources, commercial and other general and administrative functions. Other general and administrative expenses include an allocation of the Company's facility- and technology-related costs and professional fees for legal, consulting and accounting services.

Advertising costs are included in selling, general and administrative expenses and are expensed as incurred. The Company considers advertising costs as expenses related to the promotion of the Company's commercial products. For the years ended December 31, 2024, 2023 and 2022, advertising costs totaled $32.0 million, $28.6 million and $14.6 million, respectively.

Stock-Based Compensation

The Company's stock-based compensation programs include stock options, restricted stock units ("RSUs"), RSU shares with performance conditions ("PSUs") and an employee stock purchase program ("ESPP"). Stock-based compensation is recognized based on grant date fair value of stock awards.

The fair value of stock options are estimated on the date of grant using the Black-Scholes-Merton option-pricing model. The fair values of PSUs and RSUs are based on the fair market value of the Company's common stock on the date of the grant. The fair value of stock awards, with consideration given to estimated forfeitures, is recognized as stock-based compensation expense on a straight-line basis over the requisite service period, which is the vesting period of the grants. The Company estimates forfeitures over the requisite service period using historical forfeiture activity. For stock awards with performance-vesting conditions, the Company does not recognize compensation expense until it is probable that the performance condition will be achieved.

Additionally, the Company granted its CEO options with service and market conditions. A market condition relates to the achievement of a specified price of the Company's common stock, a specified amount of intrinsic value indexed to the Company's common stock or a specified price of the Company's common stock in terms of other similar equity shares. The grant date fair value for the options with service and market conditions is determined by a lattice model with Monte Carlo simulations and is recognized as stock-based compensation expense on a straight-line basis over the respective derived service period.

Under the Company's ESPP, participating employees purchase common stock through payroll deductions. The purchase price is equal to 85% of the lower of the closing price of the Company's common stock on the first business day and the last business day of the relevant purchase period. The fair value of stock purchase rights is estimated using the Black-Scholes-Merton option-pricing model. The fair value of the look-back provision with the 15% discount is recognized on a graded-vesting basis as stock-based compensation expense over the offering period.

Income Taxes

The Company follows the asset and liability method of accounting for income taxes, which requires the recognition of deferred tax assets and liabilities for expected future tax consequences attributable to differences between the consolidated financial statement carrying amounts of existing assets and liabilities and their respective tax bases. Deferred tax assets and liabilities are measured using enacted tax rates in effect for the year in which the differences are expected to reverse. A valuation allowance is recorded to reduce the net deferred tax asset to zero when it is more likely than not that the net deferred tax asset will not be realized.

The Company recognizes the effect of income tax positions only if those positions are more likely than not of being sustained upon an examination. The amount of the benefit that may be recognized in the financial statements is the largest amount that has a greater than 50% likelihood of being realized. The Company recognizes interest and penalties related to uncertain tax positions within income tax expense.

It is the intention of the Company to reinvest the earnings of its non-U.S. subsidiaries in those operations and not to repatriate the earnings to the U.S. Accordingly, the Company does not provide for deferred taxes on the excess of the financial reporting over the tax basis in its investments in foreign subsidiaries as they are considered permanent in duration.

Effective December 31, 2021, the Company adopted a policy to account for Global Intangible Low-Taxed Income ("GILTI") as a period cost under the Tax Cuts and Jobs Act. In 2021, the Organization for Economic Co-operation and Development ("OECD") released a framework for the fundamental reform of international tax rules. The framework provides for two primary "Pillars"; however, only Pillar Two, which provides for a global minimum corporate tax rate of 15%, is expected to be applicable to the Company (Pillar One is not expected to be applicable as the Company does not currently meet the turnover threshold - EUR 20 billion). In December 2022, Pillar Two was adopted by the Council of the European Union (the "EU") for implementation by EU member states by December 31, 2023, with effect for tax years beginning in calendar year 2024. The OECD, and its member countries, continue to release new guidance on these rules and the Company is continuously evaluating the impact to its financial position. Currently, the global enactment of Pillar Two did not materially impact the Company's effective tax rate or cash flows. However, the Company will continue to monitor and evaluate new legislation and guidance, which could change its current assessment.

Leases

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on the unique facts and circumstances present in the arrangement. Leases with a term greater than 12 months are recognized on the consolidated balance sheets as ROU assets and short-term and long-term lease liabilities, as applicable. The Company has elected not to recognize leases with terms of 12 months or less on the consolidated balance sheets. The Company typically only includes an initial lease term in its assessment of a lease arrangement. Options to renew a lease are not included in the Company's assessment unless there is reasonable certainty that the Company will renew. The Company monitors its plans to renew its leases quarterly or on an as-needed basis. In addition, the Company's lease agreements generally do not contain any residual value guarantees or restrictive covenants.

Operating lease liabilities and their corresponding ROU assets are recorded based on the present value of future lease payments over the expected remaining lease term at lease commencement. The initial measurement of the lease liability is determined based on the future lease payments, which may include lease payments that depend on an index or a rate (such as the consumer price index or other market index). The Company initially measures payments based on an index or rate by using the applicable rate at lease commencement and subsequent changes in such rates are recognized as variable lease costs. Variable payments that do not depend on a rate or index are not included in the lease liability and are recognized as they are incurred. Lease costs for operating leases are recognized on a straight-line basis over the lease term as an operating expense with unrecognized variable lease payments recognized as incurred. Certain adjustments to the ROU asset may be required for items such as lease prepayments or incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rate, which reflects the fixed rate at which the Company could borrow on a collateralized basis the amount of the lease payments in the same currency, for a similar term, in a similar economic environment. Components of a lease are bifurcated between lease components and non-lease components. The fixed and in-substance fixed contract consideration identified is then allocated based on the relative standalone price to the lease and non-lease components. The Company adopted a practical expedient provided by ASC Topic 842, *Leases*, and elected to account for the lease and non-lease components together for existing classes of underlying assets and allocates the contract consideration to the lease component only. In contrast, the Company does not apply the practical expedient for leases embedded in manufacturing and supply agreements with certain of its contract manufacturing organizations and has instead allocated contract consideration between the lease and non-lease components based on their relative standalone price.

Earnings (Loss) per Share

Basic earnings (loss) per share is computed by dividing net income (loss) by the weighted-average number of shares of common stock outstanding. Diluted earnings per share is computed based on the treasury stock method for stock awards and if-converted method for convertible notes by dividing net income by the weighted-average number of shares of common stock and potentially dilutive common stock equivalents outstanding. Potential common equivalent shares are excluded if their effect is anti-dilutive.

Embedded Derivatives

The Company evaluates certain of its financial and business development transactions to determine if embedded components of these contracts meet the definition of derivative under ASC 815. In general, embedded derivatives are required to be bifurcated from the host instrument if (i) the embedded feature is not clearly and closely related to the host contract and (ii) the embedded feature, if considered a freestanding instrument, meets the definition of a derivative. The embedded derivative is reported on the consolidated balance sheets at its fair value. Any change in fair value, as determined at each period, is recorded as a component of the consolidated statements of comprehensive income (loss).

Contingent Consideration

Certain of the Company's license and collaboration agreements include future payments that are contingent upon the receipt, or receipt and subsequent sale, of a Priority Review Voucher ("PRV"). The Company has concluded that these contingent payments represent embedded derivatives. The Company records a liability for such contingent payments at fair value on the date the agreements are effective. The Company estimates the fair value of contingent consideration derivatives through a valuation model that includes an income approach based on the probability-weighted expected cash flows that incorporated industry-based probability-adjusted assumptions relating to the achievement of the milestone and thus the likelihood of making the payments. Changes in the fair value of the contingent consideration derivatives can result from changes to one or multiple assumptions, including adjustments to the discount rates, the assumed development timeline and the probability of achievement of certain regulatory milestones. At least quarterly, or upon a material change to one or more of the assumptions discussed above, the Company assesses its contingent consideration derivatives and revalues them as necessary. If a revaluation occurs, changes in the fair value of the Company's contingent consideration derivatives are recognized in the Company's consolidated statements of comprehensive income (loss). Such changes are classified as other income (loss), net, which corresponds to the classification of any gain recognized upon the actual sale of a PRV.

Commitments and Contingencies

The Company records liabilities for legal and other contingencies when information available to the Company indicates that it is probable that a liability has been incurred and the amount of loss can be reasonably estimated. Legal costs in connection with legal and other contingencies are expensed as costs are incurred as selling, general and administrative expenses.

Recent Accounting Pronouncements

Recently adopted

In November 2023, the FASB issued ASU 2023-07, *"Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures,"* which is intended to improve reportable segment disclosure requirements, primarily through enhanced disclosures about significant expenses. The amendments will require public entities to disclose significant segment expenses that are regularly provided to the CODM and included within segment profit and loss. The amendments are effective for the Company's fiscal years beginning after December 15, 2023 and interim periods beginning after December 15, 2024, with early adoption permitted, and will be applied retrospectively to all prior periods presented in the financial statements. For more information related to the Company's adoption of ASU 2023-07, please read *Note 21, Segment Information*.

Recently issued

In December 2023, FASB issued ASU 2023-09, *"Income Taxes (Topic 740): Improvements to Income Tax Disclosures."* This ASU enhances the transparency and decision usefulness of income tax disclosures, primarily related to the rate reconciliation and income taxes paid information. ASU 2023-09 is effective for fiscal years beginning after December 15, 2024 and may be adopted on a prospective or retrospective basis. Early adoption is permitted. The Company is evaluating the impact of this guidance on its consolidated financial statements and related disclosures.

In March 2024, the SEC issued a final rule under SEC Release Nos. 33-11275 and 34-99678, *"The Enhancement and Standardization of Climate-Related Disclosures for Investors."* The rule requires disclosure of material climate-related information outside of the audited financial statements and disclosure in the footnotes addressing specified financial statement effects of severe weather events and other natural conditions above certain financial thresholds, certain carbon offsets and renewable energy credits or certificates. The standard is effective for the Company's Annual Report on Form 10-K for the year ended December 31, 2025. In April 2024, the SEC released an order staying this final rule pending judicial review of all the petitions challenging the rule. The Company is evaluating the impact of the rule and related litigation on its consolidated financial statements and related disclosures.

In November 2024, the FASB issued ASU 2024-03,*"Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Subtopic 220-40): Disaggregation of Income Statement Expenses,"* which requires public entities to provide disaggregated disclosure of income statement expenses. Public entities are required to disaggregate, in a tabular presentation, each relevant expense caption on the face of the consolidated statements of comprehensive income (loss) such as the following expenses: purchases of inventory, employee compensation, intangible asset amortization, and depreciation. ASU 2024-03 is effective for annual reporting periods beginning after December 15, 2026 and interim periods beginning after December 15, 2027, with early adoption permitted. The Company is evaluating the impact of this guidance on its consolidated financial statements and related disclosures.

3. LICENSE AND COLLABORATION AGREEMENTS

Arrowhead Pharmaceuticals, Inc.

On November 25, 2024, the Company entered into an exclusive global licensing and collaboration agreement and a stock purchase agreement (collectively, the "Arrowhead Agreement") with Arrowhead Pharmaceuticals, Inc. ("Arrowhead"), providing the Company with exclusive global rights to multiple clinical, preclinical and discovery-stage programs for rare, genetic diseases of the muscle, CNS and the lungs.

The closing of the transaction was subject to the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 and other customary conditions. The agreement became effective as of February 7, 2025 (the "Effective Date").

When the Arrowhead Agreement became effective, the Company paid Arrowhead an up-front payment of $500.0 million and invested $325.0 million in Arrowhead's common stock at a premium to the valuation on the closing date. As of the date of this report, the Company expects to record between approximately $500.0 million to $600.0 million in research and development expense for the three months ended March 31, 2025, related to those two payments to Arrowhead. Additionally, the Company will pay Arrowhead a total of $250.0 million in annual installments of $50.0 million over five years, with the first payment due on the first anniversary of the Effective Date. The Company may be obligated to make payments to Arrowhead totaling up to $10.3 billion upon the achievement of certain development, regulatory and sales milestones, inclusive of $300.0 million in near-term payments associated with the continued

enrollment of certain cohorts of a Phase 1/2 study. Furthermore, upon commercialization, the Company will be required to make tiered royalty payments based on net sales.

For the year ended December 31, 2024, there was no accounting impact as a result of the execution of the Arrowhead Agreement because the closing of the transaction did not occur until subsequent to year-end.

F. Hoffman-La Roche Ltd.

On December 21, 2019, the Company entered into a license, collaboration and option agreement with Roche and a stock purchase agreement (collectively, the "Roche Agreement") with Roche, providing Roche with exclusive commercial rights to ELEVIDYS outside the U.S. The Company retains all rights to ELEVIDYS in the U.S. and will perform all development activities within the joint global development plan necessary to obtain and maintain regulatory approvals for ELEVIDYS in the U.S. and the EU, unless otherwise agreed to by the parties. Further: (i) research and development expenses incurred under the joint global development plan will be equally shared between the Company and Roche, (ii) Roche is solely responsible for all costs incurred in connection with any development activities (other than those within the joint global development plan) that are necessary to obtain or maintain regulatory approvals outside the U.S, and (iii) the Company will continue to be responsible for the manufacturing of clinical and commercial supplies of ELEVIDYS. The Company has also granted Roche options to acquire ex-U.S. rights to certain future Duchenne-specific programs (the "Options") in exchange for separate option exercise payments, milestone and royalty considerations, and cost-sharing provisions. The agreement became effective on February 4, 2020. The Roche Agreement is governed by a joint steering committee ("JSC") formed by representatives from Roche and the Company. The JSC, among other activities, manages the overall strategic alignment between the parties, approves any material update to the joint global development plan and budget and oversees the operations of the subcommittees.

The Company received an aggregate of approximately $1.2 billion in cash consideration from Roche, consisting of an up-front payment and an equity investment in the Company. The Company may receive up to $1.7 billion in development, regulatory and sales milestones related to ELEVIDYS. The Company receives tiered royalty payments on net sales of ELEVIDYS outside of the U.S. based on the average cost to manufacture ELEVIDYS. Of the $1.2 billion up-front cash received from Roche, (i) $312.1 million, net of issuance costs, was allocated to the approximately 2.5 million shares of the Company's common stock issued to Roche based on the closing price when the shares were issued, (ii) $485.0 million was allocated to the Options, and (iii) $348.7 million was allocated to a single, combined performance obligation ("Combined Performance Obligation") comprised of: (i) the license of IP relating to ELEVIDYS transferred to Roche, (ii) the related research and development services provided under the joint global development plan, (iii) the services provided to manufacture clinical supplies of ELEVIDYS, and (iv) the Company's participation in the JSC, because the Company determined that the license of IP and related activities were not capable of being distinct from one another.

The value assigned to the Options is reflected as deferred revenue and will not be recognized until an option is either: (i) exercised by Roche, or (ii) expires. If exercised, the value will be aggregated with the option exercise price and recognized over the applicable performance period. If expired, the value will be recognized immediately. The Company recognizes revenue related to the Combined Performance Obligation on a straight-line basis over the expected performance period of the joint global development plan, which extended through the fourth quarter of 2023. Revenue relating to future development, regulatory and sales milestones will be recognized when the milestone is probable of achievement (which is typically when the milestone has occurred). Any royalties payable by Roche will be recognized in the period earned.

For the years ended December 31, 2024, 2023 and 2022, the Company recognized $114.0 million, $98.5 million and $89.2 million of collaboration and other revenues, respectively, which primarily consists of collaboration revenue, contract manufacturing revenue and royalty revenue related to the Roche Agreement.

On February 12, 2024, Roche declined to exercise a certain Option related to one external, early-stage development program, which resulted in that Option's expiry and the immediate recognition of the value assigned to that Option as collaboration revenue. As such, the Company recognized $48.0 million of collaboration revenue during the year ended December 31, 2024. As of December 31, 2024 and 2023, the Company had total deferred revenue of $455.3 million and $487.4 million, respectively, which is primarily associated with the Roche Agreement, of which $130.3 million and $50.4 million was classified as current. On February 5, 2025, an option for a certain program expired, which resulted in the immediate recognition of the value assigned to that option, $112.0 million, as collaboration revenue during the three months ended March 31, 2025. As of December 31, 2024, the amount is classified as deferred revenue, current portion, on the Company's consolidated balance sheets.

For both the years ended December 31, 2023 and 2022, the Company recognized $89.2 million of collaboration revenue, which relates to the amortization of the Combined Performance Obligation. The fair value allocated to Combined Performance Obligation was fully amortized as of December 31, 2023.

In accordance with the Roche Agreement, the parties agreed to enter into a supply agreement with Roche in order to supply them with clinical and commercial batches of ELEVIDYS (the "Supply Agreement"). Roche utilizes the supply for sales of ELEVIDYS in territories outside of the U.S where Roche has received certain approvals for ELEVIDYS. The Company is eligible to receive royalties on these sales. While the Supply Agreement is in the process of being negotiated at the issuance of this annual report, the Company delivered several batches of commercial ELEVIDYS supply to Roche that were agreed upon on a purchase order-by-purchase order basis. Contract manufacturing revenue and royalty revenue are included in collaboration and other revenues in the accompanying consolidated statements of comprehensive income (loss). The following table summarizes certain Roche activity for each of the periods indicated. There was no similar activity for the year ended 2022.

	For the Year Ended December 31,	
	2024	2023
	(in thousands)	
Contract manufacturing revenue	$ 49,038	$ 9,216
Royalty revenue	16,981	—
Cost of sales (inventory costs related to products sold to Roche)	(22,247)	(1,818)

The costs associated with co-development activities performed under the Roche Agreement are included in operating expenses, with any reimbursement of costs by Roche reflected as a reduction of such expenses when the related expense is incurred. For the years ended December 31, 2024, 2023 and 2022, costs reimbursable by Roche and reflected as a reduction to operating expenses were $127.1 million, $106.9 million and $117.8 million, respectively. As of December 31, 2024 and 2023, there were $34.6 million and $29.8 million of collaboration and other receivables included in other current assets on the consolidated balance sheets, respectively.

Genethon

The Company entered into a sponsored research agreement in May 2017 and subsequently a license and collaboration agreement with Genethon in November 2019 (the "Genethon Collaboration Agreement") for Genethon's micro-dystrophin gene therapy program for the treatment of Duchenne. The Genethon Collaboration Agreement grants the Company with exclusive rights in the majority of the world (primarily excluding the EU) to Genethon's micro-dystrophin gene therapy products ("Genethon Products") and other micro-dystrophin gene therapy products.

Upon signing the Genethon Collaboration Agreement, the Company made an up-front payment of $28.0 million, which was recorded as research and development expense in the Company's consolidated statements of comprehensive loss for the year ended December 31, 2019. Additionally, for the years ended December 31, 2024, 2023 and 2022, the Company recorded $6.8 million, $6.6 million and $3.5 million, respectively, of research and development expense related to reimbursable development costs incurred by Genethon for Genethon Products. For the years ended December 31, 2024, 2023 and 2022, there were no development or regulatory milestones deemed probable of being achieved and, accordingly, no additional expense has been recognized.

During May 2024, the Company and Genethon agreed to terminate the Genethon Collaboration Agreement, effective June 2024. This resulted in the elimination of all future shared development costs and future development, regulatory and sales milestone obligations.

Myonexus Therapeutics Inc.

In April 2019, the Company completed its acquisition of Myonexus Therapeutics, Inc. ("Myonexus"), a clinical-stage gene therapy biotechnology company that was developing gene therapies for LGMD for $178.3 million. The Company may also be required to make up to $200.0 million in additional payments to selling shareholders of Myonexus based on the achievement of certain sales-and regulatory- related milestones. The acquisition was accounted for as an asset acquisition as substantially all of the fair value of the gross assets acquired is concentrated in a group of similar identifiable assets (the five LGMD gene therapy programs). As part of the consideration for the transaction, the Company is required to make contingent payments to the selling shareholders of Myonexus upon the receipt and subsequent sale of a PRV with respect to a Myonexus product. As of December 31, 2024 and 2023, the contingent consideration liability was $47.1 million and $37.7 million, respectively. The changes in fair value are recorded within other income (expense), net, in the Company's consolidated statements of comprehensive income (loss). Please read *Note 5, Fair Value Measurements* for further information on the change in fair value of the contingent consideration liability.

Nationwide Children's Hospital

In December 2016, the Company entered into an exclusive option agreement with Nationwide from which the Company obtained an exclusive right to acquire a worldwide license of the micro-dystrophin gene therapy technology for Duchenne and Becker

muscular dystrophy. In October 2018, the Company exercised the option and entered into a license agreement with Nationwide, which granted the Company exclusive worldwide rights to develop, manufacture and commercialize a micro-dystrophin gene therapy product candidate. Under this agreement, the Company is liable for future regulatory milestone, sales milestone and sublicense payments as well as lower single-digit royalties upon commercialization.

During the year ended December 31, 2023, the Company recorded $23.0 million as an in-licensed right intangible asset in its consolidated balance sheets, $10.0 million of which related to the regulatory approval of ELEVIDYS, and the remaining related to sales-based milestones as the Company determined that all sales-based milestones were achieved or were probable of being achieved. As of December 31, 2024, the in-licensed right asset with a net carrying value of $20.6 million is being amortized on a straight-line basis over the remaining life of the relevant patent. Royalty payments due to Nationwide associated with commercial sales of ELEVIDYS totaled $30.6 million and $6.0 million for the years ended December 31, 2024 and 2023, respectively, and were recorded as cost of sales in the accompanying consolidated statements of comprehensive income (loss).

BioMarin Pharmaceutical, Inc.

In July 2017, the Company and UWA entered into a settlement agreement with BioMarin, and simultaneously entered into a license agreement, which was subsequently amended in April 2019 (the "BioMarin Agreement"), with BioMarin and Academisch Ziekenhuis Leiden (collectively with the Company, UWA and BioMarin, the "Settlement Parties"). The BioMarin Agreement provides the Company with an exclusive license to certain intellectual property with an option to convert the exclusive license into a co-exclusive license and the Settlement Parties agreed to stop most existing efforts to continue with ongoing litigation and opposition and other administrative proceedings concerning BioMarin's intellectual property. BioMarin is also eligible to receive tiered royalty payments, ranging from 4% to 8%, based on the net sales for the three products and product candidates.

In November 2021, the Company entered into a second settlement agreement and second amendment to the license agreement (the "Second Amendment"), which waived certain future milestone payments and altered royalty payment terms of the agreement. Under the Second Amendment, the Company may be liable for up to approximately $50.0 million in regulatory milestones for eteplirsen, casimersen and golodirsen. In addition, on and after July 1, 2022, the tiered royalty payments ranged from 4% to 5%. The royalty terms under the license agreement expired in March 2024 in the U.S. and expired as of December 31, 2024 in the EU and other countries.

As a result of the execution of the license agreement with BioMarin, the Company recorded an in-licensed right intangible asset of $6.6 million in its consolidated balance sheets as of December 31, 2017, representing the fair value of the U.S. license to BioMarin's intellectual property. The intangible asset is being amortized on a straight-line basis over the remaining life of the patent and has a carrying value of $1.8 million as of December 31, 2024.

For the years ended December 31, 2024, 2023 and 2022, the Company recognized royalty expense of $4.4 million, $17.6 million and $30.4 million, respectively, which is included in cost of sales in the accompanying consolidated statements of comprehensive income (loss). For the years ended December 31, 2024, 2023 and 2022, no regulatory milestones were deemed probable of being achieved and, accordingly, no additional in-licensed rights or expenses have been recognized.

University of Western Australia

In April 2013, the Company and UWA entered into an amendment to an existing exclusive license agreement relating to the treatment of Duchenne by inducing the skipping of certain exons. The agreement was further amended in June 2016. Under the amended agreement, the Company may be obligated to make payments to UWA totaling up to $26.0 million upon the achievement of certain development, regulatory and sales milestones. Additionally, the Company is required to pay a low-single-digit percentage royalty on net sales of products covered by issued patents licensed under the agreements with UWA. Corresponding to the FDA approval of EXONDYS 51 in 2016, VYONDYS 53 in 2019, and AMONDYS 45 in 2021, the Company recorded milestone payments of $1.0 million, $0.5 million and $0.5 million as in-licensed right intangible assets in its consolidated balance sheets, respectively. Each in-licensed right is being amortized on a straight-line basis over the remaining life of the relevant patents and have a combined carrying value of $0.5 million as of December 31, 2024. For the years ended December 31, 2024, 2023 and 2022, the Company recorded $12.1 million, $11.8 million and $10.5 million in royalty expense, respectively, which is included in cost of sales, related to agreements with UWA. For the years ended December 31, 2024, 2023 and 2022, no development, regulatory or sales milestones were deemed probable of being achieved and, accordingly, no additional in-licensed rights or expenses have been recognized.

Research and Option Agreements

The Company has research and option agreements with third parties in order to develop various technologies and biologics that may be used in the administration of the Company's genetic therapeutics. The agreements generally provide for research services related to pre-clinical development programs and options to license the technology for clinical development. Prior to the options under these agreements being executed, the Company may be required to make up to $34.8 million in research milestone payments. Under these agreements, there are $116.8 million in potential option payments to be made by the Company upon the determination to exercise the options. Additionally, if the options for each agreement are executed, the Company would incur additional contingent obligations and may be required to make development, regulatory, and sales milestone payments and royalty payments based on the sales of the developed products upon commercialization.

For the years ended December 31, 2023 and 2022, the Company recognized $5.1 million, and $6.0 million in research, option and milestone expense as research and development expense in the accompanying consolidated statements of comprehensive income (loss), respectively, with no similar activity during the year ended December 31, 2024. For the years ended December 31, 2023 and 2022, the Company exercised options in a research and option agreement and recognized $7.5 million and $8.5 million, respectively, of up-front expense as research and development expense in the accompanying consolidated statements of comprehensive income (loss), separately from the research, option and milestone expense listed above, with no similar activity for the year ended December 31, 2024.

Milestone Obligations

Including the agreements discussed above, the Company has license and collaboration agreements in place for which it could be obligated to pay, in addition to the payment of up-front fees upon execution of the agreements, certain milestone payments as a product candidate proceeds from the submission of an investigational new drug application through approval for commercial sale and beyond. As of December 31, 2024, the Company may be obligated to make up to $2.3 billion of future development, regulatory, commercial, and up-front royalty payments associated with its collaboration and license agreements. These obligations exclude potential future option and milestone payments for options that have yet to be exercised within agreements entered into by the Company as of December 31, 2024, as well as the Arrowhead Agreement that had not yet closed as of December 31, 2024, which are discussed above. For the years ended December 31, 2023 and 2022, the Company recognized approximately $13.2 million and $32.6 million relating to certain up-front, milestone, settlement and other payments as research and development expense, respectively, under these agreements, with no similar activity for the year ended December 31, 2024.

4. GAIN FROM SALE OF PRIORITY REVIEW VOUCHER

In June 2023, the Company entered into an agreement to sell the rare pediatric disease Priority Review Voucher (the "ELEVIDYS PRV") it received from the FDA in connection with the approval of ELEVIDYS for consideration of $102.0 million, with no commission costs. The closing of the transaction was not subject to the conditions set forth under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and closed in June 2023. The net proceeds were recorded as a gain from sale of the ELEVIDYS PRV during the year ended December 31, 2023, as it did not have a carrying value at the time of the sale.

5. FAIR VALUE MEASUREMENTS

The tables below present information about the Company's financial assets and liabilities that are measured and carried at fair value and indicate the level within the fair value hierarchy of the valuation techniques it utilizes to determine such fair value:

	Fair Value Measurement as of December 31, 2024			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 455,535	$ 455,535	$ —	$ —
Government and government agency bonds	279,899	—	279,899	—
Corporate bonds	93,727	—	93,727	—
Strategic investments	3,710	2,710	—	1,000
Certificates of deposit	11,319	—	11,319	—
Total assets	$ 844,190	$ 458,245	$ 384,945	$ 1,000
Liabilities				
Contingent consideration	$ 47,400	$ —	$ —	$ 47,400
Total liabilities	$ 47,400	$ —	$ —	$ 47,400

	Fair Value Measurement as of December 31, 2023			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 63,919	$ 63,919	$ —	$ —
Commercial paper	113,362	—	113,362	—
Government and government agency bonds	1,001,137	—	1,001,137	—
Corporate bonds	130,380	—	130,380	—
Strategic investments	6,527	5,527	—	1,000
Certificates of deposit	56,621	—	56,621	—
Total assets	$ 1,371,946	$ 69,446	$ 1,301,500	$ 1,000
Liabilities				
Contingent consideration	$ 38,100	$ —	$ —	$ 38,100
Total liabilities	$ 38,100	$ —	$ —	$ 38,100

The Company's assets with a fair value categorized as Level 1 within the fair value hierarchy include money market funds and the Company's strategic investment in a biotechnology company listed on the Nasdaq Global Market (the "Nasdaq"). The Company's $2.7 million strategic investment is included in other non-current assets in the Company's consolidated balance sheets, with changes in fair value being recorded within other income (expense), net in the consolidated statements of comprehensive income (loss).

The Company's assets with a fair value categorized as Level 2 within the fair value hierarchy consist of commercial paper, government and government agency bonds, corporate bonds and certificates of deposit. The Company's commercial paper, government and government agency bonds, corporate bonds and certificates of deposit have been initially valued at the transaction price and subsequently valued at the end of each reporting period utilizing third-party pricing services. The Company uses observable market inputs to determine value, which primarily consist of reportable trades. Certain short-term investments with maturities of less than three months at the date of acquisition are presented as cash equivalents on the consolidated balance sheets as of December 31, 2024 and 2023.

The Company's assets with a fair value categorized as Level 3 within the fair value hierarchy consist of a strategic investment in a private biotechnology company whose fair value measurement was based upon significant inputs not observable in the market and therefore represented a Level 3 measurement.

The Company's contingent consideration liability with a fair value categorized as Level 3 within the fair value hierarchy relates to the regulatory-related contingent payments to Myonexus selling shareholders as well as to an academic institution under a separate license agreement that meets the definition of a derivative. For more information related to Myonexus, please read *Note 3, License and Collaboration Agreements*. The contingent consideration liability was estimated using an income approach based on the probability-weighted expected cash flows that incorporated industry-based probability adjusted assumptions relating to the achievement of the milestone and thus the likelihood of making the payments. This fair value measurement was based upon significant inputs not observable in the market and therefore represented a Level 3 measurement. Significant changes, which increase or decrease the probabilities of achieving the milestone or shorten or lengthen the time required to achieve the milestone, would result in a corresponding increase or decrease in the fair value of the liability. At the end of each reporting period, the fair value is adjusted to reflect the most current assumptions through earnings.

The Company assesses its financial assets measured at fair value on a recurring basis and transfers its financial assets between the relevant fair value hierarchies at the end of each reporting period, as needed. There were no transfers into or out of Level 3 during the year ended December 31, 2024. During the year ended December 31, 2023, the Company's strategic investment in a formerly private biotechnology company transferred into Level 1 from Level 3 as a result of the biotechnology company's listing on the Nasdaq. The following table represents a roll-forward of the fair value of Level 3 financial assets for each of the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Fair value, beginning of year	$ 1,000	$ 31,000
Additions	—	4,000
Transfers out of Level 3	—	(4,000)
Changes in estimated fair value	—	(30,000)
Fair value, end of year	$ 1,000	$ 1,000

At the end of each reporting period, the fair value of the Company's strategic investments that are not publicly traded securities are adjusted if the issuers issue similar or identical securities or when there is a triggering event for impairment. There were no valuation measurement events related to the fair value of the Company's Level 3 strategic investments during the year ended December 31, 2024, as no impairment indicators were identified nor were similar securities issued. During the year ended December 31, 2023, the Company impaired its investment in Series A preferred stock of Lacerta Therapeutics, Inc. ("Lacerta") after comparing the fair value of the Lacerta strategic investment to its carrying value, resulting in an impairment loss of $30.0 million. The Company's assessment considered entity-specific impairment indicators, such as the future business prospects of Lacerta's existing programs and expected future cash flows. The fair value as of December 31, 2023 was estimated based on the expectation that the Company would realize no future cash flows associated with its investment in Lacerta. The impairment loss is included in other income (expense), net within the consolidated statements of comprehensive income (loss). During the year ended December 31, 2022, the company recorded impairment losses of $2.6 million related to its strategic investments.

The following table represents a roll-forward of the fair value of Level 3 financial liabilities for each of the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Fair value, beginning of year	$ 38,100	$ 36,900
Change in estimated fair value	9,500	2,000
Liabilities terminated	(200)	(800)
Fair value, end of year	$ 47,400	$ 38,100

A net increase of $9.3 million and $1.2 million were recorded in other income (expense), net in the consolidated statement of comprehensive income (loss) during the years ended December 31, 2024 and 2023, respectively, to account for the change in fair value and termination of liabilities of the Company's existing contingent consideration liabilities. These changes were a result of updates made to certain inputs and assumptions impacting the probability-weighted expected cash flows, principally the probability of success of the underlying programs and the discount rate utilized to determine the present value of future payments to be made, partially offset by the termination of a license agreement with an academic institution that included an embedded derivative during both years ended December 31, 2024 and 2023. As of December 31, 2024 and 2023, the remaining contingent consideration was recorded as a non-current liability on the Company's consolidated balance sheets.

The carrying amounts reported in the consolidated balance sheets for cash and cash equivalents, accounts receivable, net and accounts payable approximated fair value because of the immediate or short-term maturity of these financial instruments. For fair value information related to the Company's debt facilities, please read *Note 13, Indebtedness*.

6. CASH, CASH EQUIVALENTS AND MARKETABLE SECURITIES

The following table summarizes the Company's financial assets with maturities of less than 90 days from the date of purchase included in cash equivalents in the consolidated balance sheets for each of the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Money market funds	$ 455,535	$ 63,919
Commercial paper	—	53,680
Total	$ 455,535	$ 117,599

It is the Company's policy to mitigate credit risk in its financial assets by maintaining a well-diversified portfolio that limits the amount of exposure as to maturity and investment type. The weighted-average maturity of the Company's available-for-sale securities was approximately eleven and five months as of December 31, 2024 and 2023, respectively. All of the Company's non-current investments as of December 31, 2024 had maturities between one and two years.

The following tables summarize the Company's cash, cash equivalents, short-term investments and non-current investments for each of the periods indicated:

	As of December 31, 2024			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 1,103,010	$ —	$ —	$ 1,103,010
Government and government agency bonds	280,001	227	(329)	279,899
Corporate bonds	93,816	67	(156)	93,727
Certificates of deposit	11,319	—	—	11,319
Total cash, cash equivalents and investments	$ 1,488,146	$ 294	$ (485)	$ 1,487,955
As reported:				
Cash and cash equivalents	$ 1,103,010	$ —	$ —	$ 1,103,010
Short-term investments	251,598	286	(102)	251,782
Non-current investments	133,538	8	(383)	133,163
Total cash, cash equivalents and investments	$ 1,488,146	$ 294	$ (485)	$ 1,487,955

	As of December 31, 2023			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 374,750	$ —	$ —	$ 374,750
Commercial paper	113,362	—	—	113,362
Government and government agency bonds	1,000,302	1,006	(171)	1,001,137
Corporate bonds	130,270	118	(8)	130,380
Certificates of deposit	56,621	—	—	56,621
Total cash, cash equivalents and investments	$ 1,675,305	$ 1,124	$ (179)	$ 1,676,250
As reported:				
Cash and cash equivalents	$ 428,430	$ —	$ —	$ 428,430
Short-term investments	1,246,875	1,124	(179)	1,247,820
Total cash, cash equivalents and investments	$ 1,675,305	$ 1,124	$ (179)	$ 1,676,250

7. PRODUCT REVENUES, NET, ACCOUNTS RECEIVABLE, NET AND RESERVES FOR PRODUCT REVENUES

Net product revenues, which includes revenues associated with the PMO Products and ELEVIDYS, consisted of the following:

	For the Year Ended December 31,		
	2024	2023	2022
		(in thousands)	
PMO Products			
United States	$ 816,920	$ 797,944	$ 747,101
Rest of World	150,249	146,576	96,668
Total PMO product revenues, net	$ 967,169	$ 944,520	$ 843,769
ELEVIDYS			
United States	820,791	200,356	—
Total ELEVIDYS product revenues, net	$ 820,791	$ 200,356	$ —
Total product revenues, net	$ 1,787,960	$ 1,144,876	$ 843,769

No individual country outside the U.S. exceeded 10% of total net product revenues for any of the years ended December 31, 2024, 2023 and 2022.

The following table summarizes the Company's net product revenues, by customer, for those customers that exceeded 10% for the periods indicated:

	For the Year Ended December 31,		
	2024	2023	2022
Product revenues, net			
Customer 1	31%	41%	48%
Customer 2	17%	26%	33%

As of December 31, 2024 and 2023, the Company's accounts receivable, net were $602.0 million and $400.3 million, respectively, which related to product sales, net of discounts and allowances. Please refer to *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* for discussion of the credit risk associated with accounts receivable, net.

The following table summarizes an analysis of the change in reserves for discounts and allowances for the periods indicated:

	Chargebacks	Rebates	Prompt Pay	Other Accruals	Total
			(in thousands)		
Balance, as of December 31, 2022	$ 417	$ 67,493	$ 3,343	$ 23,445	$ 94,698
Provision	44,191	129,724	15,067	69,664	258,646
Adjustments relating to prior year	536	(5,103)	—	—	(4,567)
Payments/credits	(17,658)	(93,920)	(14,579)	(57,848)	(184,005)
Balance, as of December 31, 2023	$ 27,486	$ 98,194	$ 3,831	$ 35,261	$ 164,772
Provision	130,180	169,244	19,574	80,398	399,396
Adjustments relating to prior year	783	(19,732)	—	(88)	(19,037)
Payments/credits	(112,545)	(139,863)	(17,464)	(80,960)	(350,832)
Balance, as of December 31, 2024	$ 45,904	$ 107,843	$ 5,941	$ 34,611	$ 194,299

The following table summarizes the total reserves above included in the Company’s consolidated balance sheets for the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Reduction to accounts receivable, net	$ 85,142	$ 64,697
Component of accrued expenses	109,157	100,075
Total reserves	$ 194,299	$ 164,772

8. INVENTORY

The following table summarizes the components of the Company’s inventory for each of the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Raw materials	$ 280,045	$ 133,963
Work in progress	610,692	318,458
Finished goods	47,209	61,806
Total inventory	$ 937,946	$ 514,227

No material inventory reserves existed as of December 31, 2024 or 2023. The Company classifies inventory associated with its PMO Products as non-current inventory when consumption of the inventory is expected beyond the Company's normal PMO Product inventory operating cycle of two years. Non-current inventory consists of raw materials and work in progress associated with the PMO Products.

The following table summarizes the balance sheet classification of the Company's inventory for each of the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Balance sheet classification		
Inventory	$ 749,960	$ 322,859
Non-current inventory	187,986	191,368
Total inventory	$ 937,946	$ 514,227

9. OTHER ASSETS

The following table summarizes the Company's other current assets for each of the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Collaboration and other receivables	$ 34,608	$ 29,786
Prepaid maintenance services	13,407	11,281
Tax-related receivables and prepaids	13,132	6,862
Prepaid clinical and pre-clinical expenses	10,220	10,280
Prepaid insurance	3,668	3,352
Prepaid commercial expenses	3,371	2,729
Prepaid employee benefits	2,841	946
Other	9,214	12,478
Total other current assets	$ 90,461	$ 77,714

The following table summarizes the Company's other non-current assets for each of the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Intangible assets, net	$ 26,887	$ 29,620
Manufacturing-related deposits and prepaids	25,964	74,204
Restricted cash*	15,579	15,579
Prepaid maintenance services	4,381	5,466
Strategic investments	3,710	6,527
Other	3,394	5,375
Total other non-current assets	$ 79,915	$ 136,771

* The Company had approximately $15.6 million in restricted cash included in other non-current assets on the Company's consolidated balance sheets as of December 31, 2024 and 2023. Restricted cash for both years relates to (i) letters of credit established under the Company's various property leases that serve as security for potential future default of lease payments, (ii) a letter of credit established under a certain commercial supply agreement and (iii) collateralized cash for the Company's credit cards. The restricted cash is unavailable for withdrawal or use for general obligations.

10. PROPERTY AND EQUIPMENT, NET

Property and equipment are recorded at historical cost, net of accumulated depreciation and accumulated impairment losses. The following table summarizes components of property and equipment, net, for each of the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Construction in progress	$ 165,901	$ 91,089
Leasehold improvements	155,073	99,989
Lab and manufacturing equipment	129,881	108,101
Building and improvements	51,178	48,063
Software and computer equipment	50,179	50,179
Furniture and fixtures	10,399	9,339
Land and land improvements	10,171	10,171
Office equipment	1,699	1,193
Property and equipment, gross	574,481	418,124
Less: accumulated depreciation	(233,704)	(190,970)
Less: accumulated impairment loss	(441)	—
Property and equipment, net	$ 340,336	$ 227,154

For the years ended December 31, 2024, 2023 and 2022, depreciation expense totaled $42.7 million, $42.5 million, and $40.0 million, respectively.

11. INTANGIBLE ASSETS, NET

The following table summarizes the components of the Company’s intangible assets for each of the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
In-licensed rights	$ 32,673	$ 32,673
Patents	4,858	4,889
Software licenses	302	302
Intangible assets, gross	37,833	37,864
Less: accumulated amortization	(10,946)	(8,244)
Intangible assets, net	$ 26,887	$ 29,620

The in-licensed rights relate to agreements with UWA, Nationwide, BioMarin and PPMD. These in-licensed rights are being amortized on a straight-line basis over the remaining life of the related patent because the life of the related patent reflects the expected time period that the Company will benefit from the in-licensed rights. There were no additions of in-licensed rights during the year ended December 31, 2024. For more information related to the Company's in-licensed rights related to its license or settlement agreements with Nationwide, BioMarin and UWA, please refer to *Note 3. License and Collaboration Agreements.* For the years ended December 31, 2024, 2023 and 2022, the Company recorded $2.7 million, $1.9 million and $1.9 million, respectively, of amortization expense related to intangible assets.

The following table summarizes the estimated future amortization for intangible assets:

	As of December 31, 2024
	(in thousands)
2025	$ 2,648
2026	2,513
2027	2,444
2028	2,366
2029	1,957
Thereafter	14,959
Total	$ 26,887

12. ACCRUED EXPENSES

The following table summarizes the Company's accrued expenses for each of the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Product revenue related reserves	$ 109,157	$ 100,075
Accrued employee compensation costs	91,119	78,732
Accrued contract manufacturing costs	77,842	33,024
Accrued clinical and pre-clinical costs	26,849	34,669
Accrued professional fees	17,691	17,187
Accrued income taxes	17,391	13,766
Accrued royalties	16,625	12,070
Accrued fixed assets	5,305	3,231
Accrued interest expense	4,192	4,395
Accrued milestone and license expense	2,000	11,375
Other	5,342	6,473
Total accrued expenses	$ 373,513	$ 314,997

13. INDEBTEDNESS

2027 Convertible Notes and Related Transactions

Issuance and Capped Call Transactions

In September 2022, the Company issued $1,150.0 million aggregate principal amount of convertible senior notes due on September 15, 2027. The 2027 Notes are senior unsecured obligations of the Company and bear interest at a rate of 1.25% per annum, payable semi-annually in cash on each March 15 and September 15, commencing on March 15, 2023. The net proceeds were $1,126.7 million after deducting the discounts and offering expenses of $23.3 million, which is amortized under the effective interest method and recorded as additional interest expense over the life of the 2027 Notes. The effective interest rate on the 2027 Notes is 1.67%. The aggregate issuance of the 2027 Notes includes the issuance of $20.0 million in aggregate principal amount of 2027 Notes to the Michael A. Chambers Living Trust, an entity affiliated with Michael Chambers, a member of the Company's board of directors.

The 2027 Notes may be convertible into 8,100,485 shares of the Company's common stock at a conversion rate of 7.0439 shares per $1,000 principal amount of the 2027 Notes, or a conversion price of $141.97 per share. Upon conversion, at its discretion, the Company may pay cash, shares of its common stock or a combination of cash and stock. Prior to March 15, 2027, the holders of the 2027 Notes may convert their 2027 Notes at their option upon achievement of certain market conditions or occurrence of certain corporate events.

Only on or after September 20, 2025, should certain market conditions be met, the Company may redeem for cash all or any portion of the 2027 Notes at a redemption price equal to the principal amount of the 2027 Notes, plus accrued and unpaid interest. Holders of the 2027 Notes have the right to require the Company to repurchase for cash all or a portion of their notes at 100% of its respective principal amount, plus any accrued and unpaid interest, upon the occurrence of a fundamental change as defined in the indenture agreement for the 2027 Notes. The 2027 Notes contain customary covenants and events of default, occurrence of which permits the holders to accelerate all outstanding obligations, including principal and interest.

In connection with the issuance of the 2027 Notes, the Company entered into privately negotiated capped call transactions with counterparties intended to minimize the impact of potential dilution upon conversion of the 2027 Notes (the "2022 Capped Calls"), which covers 8,100,485 shares of the Company's common stock. The 2022 Capped Calls have an initial strike price of approximately $141.97 per share and a cap price of approximately $210.32 per share. If, upon conversion of the 2027 Notes, the price of the Company's common stock is between the strike price and the cap price of the capped calls, the counterparties will deliver shares of the Company's common stock and/or cash with an aggregate value equal to the difference between the price of the Company's common

stock at the conversion date and the strike price, multiplied by the number of shares of the Company's common stock related to the capped calls being exercised. The Company paid $127.3 million for the 2022 Capped Calls.

Termination of 2019 Term Loan

On September 16, 2022, using proceeds received from the issuance of the 2027 Notes described above, the Company prepaid in full all of its amounts outstanding with respect to the December 2019 term loan with Biopharma Credit PLC and Biopharma Credit Investments V (Master) LP (the "December 2019 Term Loan") and repaid in full all obligations due. The aggregate payoff amount was approximately $585.5 million, which includes $550.0 million of principal, additional loan consideration and premiums of $25.4 million, and accrued interest of $10.1 million through the repayment date. The loss on debt extinguishment was $26.9 million, and was included in the loss on debt extinguishment in the consolidated statements of comprehensive income (loss) for the year ended December 31, 2022.

2024 Convertible Notes and Related Transactions

Issuance and Capped Call Transactions

In November 2017, the Company issued $570.0 million aggregate principal amount of senior convertible notes due on November 15, 2024. The 2024 Notes are senior unsecured obligations of the Company and bear interest at a rate of 1.50% per annum, payable semi-annually in cash on each May 15 and November 15, commencing on May 15, 2018. The net proceeds were $559.4 million after deducting the discounts and offering expenses of $10.6 million, which is amortized under the effective interest method and recorded as additional interest expense over the life of the 2024 Notes. The effective interest rate on the 2024 Notes is 1.9%.

The 2024 Notes could be convertible into 7,763,970 shares of the Company's common stock under certain circumstances prior to maturity at a conversion rate of 13.621 shares per $1,000 principal amount of the 2024 Notes, or a conversion price of $73.42 per share, subject to adjustment under certain conditions. Upon conversion, the Company could pay cash, shares of its common stock or a combination of cash and stock, as determined by the Company at its discretion. The holders of the 2024 Notes could convert their 2024 Notes at their option upon achievement of certain market conditions or occurrence of certain corporate events.

The 2024 Notes were not redeemable by the Company prior to the maturity date. Holders of the 2024 Notes, however, had the right to require the Company to repurchase for cash all or a portion of their notes at 100% of its respective principal amount, plus any accrued and unpaid interest, upon the occurrence of a fundamental change as defined in the indenture agreement for the 2024 Notes. The 2024 Notes contained customary covenants and events of default, occurrence of which permitted the holders to accelerate all outstanding obligations, including principal and interest.

In connection with the issuance of the 2024 Notes, the Company entered into privately negotiated capped call transactions with counterparties intended to minimize the impact of potential dilution upon conversion of the 2024 Notes (the "2017 Capped Calls") which covers 7,763,970 shares of the Company's common stock. The 2017 Capped Calls had an initial strike price of approximately $73.42 per share and a cap price of approximately $104.88 per share. If, upon conversion of the 2024 Notes, the price of the Company's common stock was between the strike price and the cap price of the capped calls, the counterparties would deliver shares of the Company's common stock and/or cash with an aggregate value equal to the difference between the price of the Company's common stock at the conversion date and the strike price, multiplied by the number of shares of the Company's common stock related to the capped calls being exercised. The Company paid $50.9 million for the 2017 Capped Calls.

Repurchase and Capped Call Settlement

In September 2022, in connection with the issuance of the 2027 Notes, the Company entered into separate, privately negotiated transactions to repurchase a portion of the outstanding 2024 Notes (the "Repurchase"). The holders exchanged $150.6 million in aggregate principal value of 2024 Notes held by them for an aggregate payment of $248.6 million for full settlement of the principal value and accrued interest on such date. The repurchase was not pursuant to the conversion privileges included in the terms of the debt at issuance and therefore was accounted for as a debt extinguishment. Accordingly, on the repurchase date, the Company recorded $98.5 million of debt extinguishment expense which was included in the loss on debt extinguishment within the consolidated statements of comprehensive income (loss) for the year ended December 31, 2022. Corresponding to the Repurchase, the related 2017 Capped Calls were terminated. As a result, the Company received $26.3 million in cash.

Exchange and Capped Call Settlement

In March 2023, the Company entered into separate, privately negotiated exchange agreements with certain holders of the outstanding 2024 Notes (the "Exchange"), which resulted in an exchange of $313.5 million in aggregate principal value of the 2024 Notes for shares of the Company's common stock. In connection with the Exchange, the Company issued approximately 4.5 million shares of the Company's common stock with a fair value of approximately $693.4 million. The Company also incurred approximately $6.9 million in third-party debt conversion costs. The exchange was not pursuant to the conversion privileges included in the terms of the debt at issuance and therefore was accounted for as a debt extinguishment and resulted in a loss on debt extinguishment of $387.3 million, inclusive of the $6.9 million in third-party debt conversion costs, which was included in the consolidated statements of

comprehensive income (loss) for the year ended December 31, 2023. Corresponding to the Exchange, the related 2017 Capped Calls were terminated. As a result, the Company received $80.6 million in cash.

Conversion and Capped Call Settlement in 2024

Upon maturity as of November 15, 2024 ("the Maturity Date"), the Company converted the remaining outstanding bonds into approximately 1.2 million shares of its common stock equivalent to an aggregate principal amount of $91.6 million. A de minimis number of bonds were not converted and, as a result, the Company paid less than $0.1 million in cash to those bondholders. As the conversions were completed under the original terms of the 2024 Notes, there is no gain or loss impact on the accompanying consolidated statements of comprehensive income (loss).

In May 2024, the Company informed holders of the 2024 Notes of its election to settle the 2024 Notes in shares, which, pursuant to the terms of the 2017 Capped Call agreements, would require the Company to settle the 2017 Capped Calls in shares. On September 16, 2024 (the "Effective Date"), the Company entered into supplements with each of the counterparties to modify the terms of each of the 2017 Capped Calls to require settlement in cash rather than in shares. As a result of the modification, the Company reclassified the fair value of the 2017 Capped Calls of $43.9 million on the Effective Date from stockholders' equity to derivative assets. As a result of the conversion, the Company ultimately received $45.3 million in cash to settle the remaining 2017 Capped Calls on November 15, 2024. The change in the fair value of the derivative assets from the Effective Date to Maturity Date was a gain of $1.5 million and recorded to other income (expense), net in the consolidated statements of comprehensive income (loss). There were no derivative assets remaining as of December 31, 2024.

Total Debt Obligations

As of December 31, 2024 and 2023, the Company recorded approximately $1,137.1 million and $1,238.0 million as debt on the consolidated balance sheets, respectively. As of December 31, 2023, the Company reclassified the balance of the 2024 Notes from long-term debt to current liabilities on its consolidated balance sheets, as the 2024 Notes matured in less than twelve months. As of December 31, 2024, all debt was long-term in nature.

The following table summarizes the Company's debt instruments for the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Principal amount of the 2027 Notes	$ 1,150,000	$ 1,150,000
Principal amount of the 2024 Notes	—	105,847
Unamortized discount - debt issuance costs of 2027 Notes	(12,876)	(17,485)
Unamortized discount - debt issuance costs of 2024 Notes	—	(364)
Total carrying value of debt instruments	$ 1,137,124	$ 1,237,998
Fair value of 2027 Notes	$ 1,254,857	$ 1,172,276
Fair value of 2024 Notes	—	144,833
Total fair value of debt instruments	$ 1,254,857	$ 1,317,109

For the years ended December 31, 2024, 2023 and 2022, the Company recorded $20.6 million, $22.0 million and $53.2 million of interest expense, respectively. Total interest expense for the years ended December 31, 2024, 2023 and 2022 is inclusive of $5.0 million, $5.2 million and $7.5 million of amortization of debt discounts, respectively. The fair values of the 2027 Notes and 2024 Notes are based on open market trades and are classified as Level 1 in the fair value hierarchy.

The following table summarizes the total principal and contractual interest payments due under the Company's debt arrangements:

	As of December 31, 2024		
	Principal	Interest	Total Payments
		(in thousands)	
2025	$ —	$ 14,375	$ 14,375
2026	—	14,375	14,375
2027	1,150,000	14,375	1,164,375
Total payments	$ 1,150,000	$ 43,125	$ 1,193,125

14. EQUITY

In November 2024, the Board of Directors approved a share repurchase program (the "2024 Repurchase Program") of up to $500.0 million of the Company's outstanding common stock over the next 18 months. Correspondingly, the Company entered into a Rule 10b-18 repurchase plan that allows it to conduct open market repurchases periodically up to the remaining authorization under the 2024 Repurchase Program. There were no share repurchases of our common stock during the year ended December 31, 2024.

Repurchased shares are held as treasury stock. The amount paid to repurchase shares will be recorded as a reduction to stockholders' equity. As treasury stock is not considered shares outstanding, it will be excluded from average common stock outstanding for both basic and diluted earnings per share.

15. STOCK-BASED COMPENSATION

In June 2013, the Company's stockholders approved the 2013 Employee Stock Purchase Plan (the "2013 ESPP") which authorized 0.3 million shares of common stock available to be issued. In June 2016, 2019 and 2023, the Company's stockholders approved an additional 0.3 million, 0.5 million and 0.3 million shares, respectively, of common stock available for issuance under the 2013 ESPP. As of December 31, 2024, 0.2 million shares of common stock remain available for future grant under the 2013 ESPP.

In June 2018, the Company's stockholders approved the 2018 Equity Incentive Plan (the "2018 Plan"). The 2018 Plan, which authorized 2.9 million shares of common stock to be issued, allows for the grant of stock options, stock appreciation rights ("SARs"), restricted stock awards ("RSAs"), RSUs, performance shares and performance units. In June 2020, June 2022 and June 2023, an additional 3.8 million, 2.5 million and 2.5 million shares of common stock, respectively, were approved by the Company's stockholders and added to the 2018 Plan. As of December 31, 2024, together with the roll-over shares from the Company's 2011 Equity Incentive Plan, 3.4 million shares of common stock remain available for future grant under the 2018 Plan.

In March 2024, the Company initiated the 2024 Employment Commencement Incentive Plan (the "2024 Plan"). The 2024 Plan, which authorized 0.6 million shares of common stock to be issued, allows for the grant of stock options, SARs, RSAs, RSUs, performance shares and performance units. In June 2024, there was an addition of 0.5 million shares to the 2024 Plan. As of December 31, 2024, 1.0 million shares of common stock remain available for future grant under the 2024 Plan.

Stock Options

In general, stock options have a ten-year term and vest over a four-year period, with one-fourth of the underlying shares vesting on the first anniversary of the grant and 1/48th of the underlying shares vesting monthly thereafter, such that the underlying shares will be fully vested on the fourth anniversary of the grant, subject to the terms of the applicable plan under which they were granted.

The fair values of stock options granted during the periods presented are measured on the date of grant using the Black-Scholes-Merton option-pricing model, with the following assumptions:

	For the Year Ended December 31,		
	2024	2023	2022
Risk-free interest rate (1)	3.5 - 4.4%	3.5 - 4.9%	1.6 - 4.2%
Expected dividend yield (2)	—	—	—
Expected term (3)	5.81 years	5.23 years	5.09 years
Expected volatility (4)	40.8 - 53.5%	46.8 - 63.2%	52.4 - 72.9%

(1) The risk-free interest rate is estimated using an average of Treasury bill interest rates over a historical period commensurate with the expected term of the option that correlates to the prevailing interest rates at the time of grant.
(2) The expected dividend yield is zero as the Company has not paid any dividends to date and does not expect to pay dividends in the future.
(3) The expected term is estimated using historical exercise behavior.
(4) The expected volatility is the implied volatility in exchange-traded options of the Company's common stock.

The amounts estimated according to the Black-Scholes-Merton option-pricing model may not be indicative of the actual values realized upon the exercise of these options by the holders.

The following table summarizes the Company's stock option activity for the period indicated:

	For the Year Ended December 31, 2024	
	Shares	Weighted-Average Exercise Price
Grants outstanding at beginning of the period	9,582,695	$ 78.57
Granted	331,920	128.89
Exercised	(850,117)	79.33
Cancelled and forfeited	(180,026)	122.38
Grants outstanding at end of the period	8,884,472	$ 79.49
Grants exercisable at end of the period	6,267,957	$ 77.66
Grants vested and expected to vest at end of the period	8,726,976	$ 78.66

The weighted-average grant date fair value per share of stock options granted during the years ended December 31, 2024, 2023 and 2022 was $67.31, $70.94 and $48.82, respectively.

	Aggregate Intrinsic Value (in thousands)	Weighted-Average Remaining Contractual Life (Years)
Options outstanding at December 31, 2024	$ 425,039	4.8
Options exercisable at December 31, 2024	$ 307,232	4.4
Options vested and expected to vest at December 31, 2024	$ 423,367	4.7

The following table summarizes the Company's shares vested and stock options exercised for each of the periods indicated:

	For the Year Ended December 31,		
	2024	2023	2022
		(in thousands)	
Aggregate grant date fair value of shares vested	$ 147,631	$ 142,692	$ 140,889
Aggregate intrinsic value of stock options exercised	$ 57,158	$ 29,711	$ 12,150

Grant Modification

In June 2017, the Company granted its CEO 3,300,000 options with service and market conditions which were subject to a five-year cliff vesting schedule. On April 19, 2022 (the "Effective Date"), the Company entered into an agreement with its CEO to modify the vesting conditions of the options (the "Amendment"). Under the Amendment, one-third of the options vested (the "Vested Tranche") on the Effective Date with no required service or market conditions. Subject to the CEO's continued service through each applicable vesting date and the compound annual growth rate of the Company's common stock exceeding that of the Nasdaq Biotech Index in varying percentages, the remaining two-thirds of the options (the "Unvested Tranche") shall vest in varying increments at any time between the Effective Date and June 26, 2025 (the "Measurement Period") when (and if) the average of the closing price of the Company's common stock during any consecutive 20 trading day period during the Measurement Period reaches certain pre-determined target stock prices. Additionally, the CEO is subject to a one-year post-exercise restriction to sell, transfer or dispose shares acquired upon the exercise of any options that vest after deduction of any shares withheld or sold to pay the applicable aggregate exercise price and/or withholding taxes. The aggregate incremental cost of the modification of the CEO's awards was $123.3 million.

During both years ended December 31, 2023 and 2022, respectively, 550,110 options relating to the Unvested Tranche became vested as they met the conditions for vesting and, accordingly, all previously unrecognized expense associated with these options was immediately recognized. For the years ended December 31, 2023 and 2022, the Company recorded $13.4 million and $109.9 million as stock-based compensation expense relating to the CEO's awards, respectively. As of December 31, 2023, the Unvested Tranche was fully expensed.

Excluding the options with market and service conditions granted to the Company's CEO, the remaining stock options granted during the periods presented in the table have only service-based criteria and vest over four years.

Restricted Stock Units

The Company grants RSUs to members of its board of directors and employees. The following table summarizes the Company's RSU activity for the period indicated:

	For the Year Ended December 31, 2024		
	Shares		Weighted-Average Grant Date Fair Value
Grants outstanding at beginning of the period	2,240,504	(1)	$ 120.17
Granted	1,409,347	(2)	129.31
Vested	(734,922)		107.93
Forfeited	(197,515)		126.19
Grants outstanding at end of the period	2,717,414		$ 127.78

(1) Included in RSUs outstanding at the beginning of the year ended December 31, 2024 are 33,000 shares of PSUs (the "March 2022 PSU") with performance conditions related to regulatory approval of certain of the Company's product candidates and 485,275 shares of PSUs (the "March 2023 PSU") with performance conditions related to regulatory approval of certain of the Company's product candidates and the achievement of a certain financial performance target.

(2) Included in RSUs granted during the year ended December 31, 2024 are 97,460 shares with performance conditions (the "March 2024 PSU"), which are related to the achievement of certain financial performance targets and regulatory approval of certain of the Company's product candidates and 24,500 shares of the March 2023 PSU, which are related to the achievement of a certain financial performance target.

Stock options and the remaining RSUs granted during the year ended December 31, 2024 have only service-based criteria and vest over four years.

As of December 31, 2024, the following PSUs became vested or eligible for vesting:

- March 2022 PSU: The expanded regulatory approval of ELEVIDYS in June 2024 resulted in 33,000 shares becoming eligible for vesting, which is contingent on the fulfillment of remaining service conditions.
- March 2023 PSU: The achievement of a certain financial performance target during the year ended December 31, 2024 resulted in 24,500 shares becoming eligible for vesting, which is contingent on the fulfillment of remaining service conditions.
- March 2024 PSU: The expanded regulatory approval of ELEVIDYS in June 2024 resulted in the cliff-vesting of 44,300 of the 97,460 shares of the March 2024 PSU during the year ended December 31, 2024.

As of December 31, 2024, none of the remaining performance conditions associated with the March 2022 PSU and March 2024 PSU were probable of being achieved.

The weighted-average grant date fair value of RSUs granted during the years ended December 31, 2023 and 2022 was $151.20 and $85.39, respectively. The fair values of RSUs vested during the years ended December 31, 2024, 2023 and 2022 totaled $86.9 million, $82.6 million and $33.1 million, respectively.

2013 Employee Stock Purchase Plan

Under the Company's 2013 ESPP, participating employees purchase common stock through payroll deductions. The purchase price is equal to 85% of the lower of the closing price of the Company's common stock on the first business day and the last business day of the relevant purchase period. The 24-month offering period will end between August 31, 2025 and August 31, 2026. The following table summarizes the Company's ESPP activity for each of the periods indicated:

	For the Year Ended December 31,		
	2024	2023	2022
Number of shares purchased	143,589	153,027	115,124
Proceeds received (in millions)	$ 12.2	$ 10.8	$ 7.5

Stock-based Compensation Expense

The following table summarizes stock-based compensation expense by grant type and by function included within the consolidated statements of comprehensive income (loss):

	For the Year Ended December 31,		
	2024	2023	2022
		(in thousands)	
Stock options	$ 62,255	$ 79,472	$ 174,868
Restricted stock units	135,612	97,808	52,601
Employee stock purchase plan	7,266	5,234	5,549
Subtotal	$ 205,133	$ 182,514	$ 233,018
Capitalized stock-based compensation costs*	(20,833)	—	—
Total stock-based compensation expense included in expenses	$ 184,300	$ 182,514	$ 233,018
Research and development	74,010	82,489	61,293
Selling, general and administrative	110,290	100,025	171,725
Total stock-based compensation expense included in expenses	$ 184,300	$ 182,514	$ 233,018

*Prior to the year ended December 31, 2024, capitalized stock-based compensation costs were not material.

As of December 31, 2024, there was $238.5 million of total unrecognized stock-based compensation expense related to the Company's stock-based compensation plans, including estimated forfeitures. The expense is expected to be recognized over a weighted-average period of approximately two years. Of this amount, $72.9 million related to options with service conditions only, $7.9 million related to PSUs with certain performance conditions not met and the remaining $157.7 million related to restricted stock units with service conditions only.

16. 401(K) PLAN

The Company sponsors a 401(k) Plan in the U.S. and other retirement plans ("the Plan") in the rest of the world, all of which are defined contribution plans. The Plan is available to all employees who are age 21 or older. Participants may make voluntary contributions and the Company makes matching contributions according to the Plan's matching formula. Matching contributions fully vest after one year of service for all employees. The expense related to the Plan primarily consists of the Company's matching contributions.

Expense related to the Plan totaled $9.8 million, $8.8 million and $6.5 million for the years ended December 31, 2024, 2023 and 2022, respectively.

17. OTHER INCOME (LOSS), NET

The following table summarizes other income (loss), net for the periods indicated:

	For the Year Ended December 31,		
	2024	2023	2022
		(in thousands)	
Accretion of investment discount, net	$ 41,132	$ 49,712	$ 11,235
Interest income	30,635	36,257	16,488
Interest expense	(18,391)	(22,010)	(53,248)
Change in fair value of derivatives*	(7,838)	(1,200)	6,700
Impairment of strategic investments	—	(30,321)	(2,575)
Other, net	(2,845)	617	(6,921)
Other income (expense), net	$ 42,693	$ 33,055	$ (28,321)
Loss on debt extinguishment	—	(387,329)	(125,441)
Gain from sale of Priority Review Voucher	—	102,000	—
Total other income (loss), net	$ 42,693	$ (252,274)	$ (153,762)

* Related to the change in fair value of the contingent consideration derivative liabilities and the 2017 Capped Calls derivative assets. For more information, please read *Note 5, Fair Value Measurements* and *Note 13, Indebtedness*.

18. INCOME TAXES

The following table summarizes the income (loss) before the provision for income taxes by jurisdiction for the periods indicated:

	For the Year Ended December 31,		
	2024	2023	2022
		(in thousands)	
Domestic	$ 168,666	$ (238,660)	$ (251,384)
Foreign	92,108	(281,438)	(438,579)
Total	$ 260,774	$ (520,098)	$ (689,963)

The following table summarizes the provision for income taxes in the accompanying consolidated financial statements for the periods indicated:

	For the Year Ended December 31,		
	2024	2023	2022
		(in thousands)	
Current provision:			
Federal	$ 5,064	$ 4,180	$ —
State	19,748	11,111	13,193
Foreign	972	753	944
Total current provision	25,784	16,044	14,137
Deferred benefit:			
Federal	—	—	—
State	—	—	—
Foreign	(249)	(165)	(612)
Total deferred benefit	(249)	(165)	(612)
Total income tax expense	$ 25,535	$ 15,879	$ 13,525

The following table summarizes the reconciliation between the Company's effective tax rate and the statutory income tax rate for each of the periods indicated:

	For the Year Ended December 31,		
	2024	2023	2022
Federal income tax rate	21.0 %	21.0 %	21.0 %
State taxes	(0.5)	(7.7)	4.1
Research and development tax credits	(8.3)	28.3	6.9
Valuation allowance	2.2	(13.7)	(14.7)
Permanent differences	0.1	(0.2)	(1.1)
Stock-based compensation	6.1	(2.0)	(1.5)
Excess benefit stock deductions	(4.3)	1.6	0.3
Foreign rate differential	(7.2)	(11.3)	(13.2)
Non-deductible repurchase premium	—	—	(2.2)
Non-deductible premium on note conversion	—	(15.3)	—
Other	0.7	(3.8)	(1.6)
Effective tax rate	9.8 %	(3.1) %	(2.0) %

The significant items impacting the Company's effective tax rate for each of the periods primarily include state taxes, research and development tax credits, valuation allowance, stock-based compensation, and foreign rate differential. For the year ended December 31, 2023, the Company's effective tax rate was also significantly impacted by the $379.9 million non-deductible premium paid on the conversion of the 2024 Notes.

The following table summarizes the deferred tax assets and liabilities for each of the periods indicated:

	As of December 31,	
	2024	2023
	(in thousands)	
Deferred tax assets:		
Net operating loss carryforwards	$ 67,594	$ 154,948
Difference in depreciation and amortization	35,805	36,611
Research and development tax credits	381,451	374,017
Stock-based compensation	107,775	94,452
Lease liabilities	48,172	33,792
Capitalized inventory	7,009	35,269
Debt discount	18,678	25,597
Capitalized research and development costs	203,416	106,534
Other	52,658	50,308
Total deferred tax assets	922,558	911,528
Deferred tax liabilities:		
Right of use asset	(31,971)	(25,800)
Debt discount	—	—
Total deferred tax liabilities	(31,971)	(25,800)
Valuation allowance	(888,335)	(883,665)
Net deferred tax assets	$ 2,252	$ 2,063

For tax years beginning on or after January 1, 2022, the Tax Cuts and Jobs Act of 2017 eliminates the option to currently deduct research and development expenses and requires taxpayers to capitalize and amortize the costs over five years for research activities performed in the United States and 15 years for research activities performed outside the United States. The requirement to capitalize research and development costs for tax purposes resulted in the Company having taxable profits and recording federal and state tax expense of $5.1 million and $19.7 million, respectively. The state tax expense of $19.7 million is primarily related to the temporary suspension of utilizing net operating loss carryforwards in certain states the Company operates in.

On a periodic basis, the Company reassesses the valuation allowance on its deferred tax assets, weighing positive and negative evidence to assess the recoverability of such deferred tax assets. In assessing the Company's ability to realize its net deferred tax assets, the Company considered various factors, including future reversals of existing taxable temporary differences, projected future taxable income, potential carryback claims and tax planning strategies to determine whether it is more likely than not that some portion or all of its net deferred tax assets will not be realized. Based upon these factors, the Company has concluded that it is more likely than not that the Company will not recognize the benefits of its net federal and state deferred tax assets. Accordingly, a full valuation allowance against the U.S. net deferred tax assets is maintained at December 31, 2024 and 2023. The Company continues to monitor its recent earnings history and it is possible that within the next 12 months, there may be sufficient positive evidence to release a portion or all of the Company's valuation allowance. The release of the valuation allowance, as well as the exact timing and the amount of such release, continue to be subject to, among other things, the Company's level of profitability, revenue growth, and expectations regarding future profitability. Release of the valuation allowance would result in a benefit to income tax expense for the period the release is recorded, which could have a material impact on net earnings. The total deferred tax asset balance subject to the valuation allowance was approximately $890.6 million as of December 31, 2024. The Company will continue to monitor the need for a full or partial valuation allowance on a quarterly basis.

The Company generated foreign deferred tax assets mainly consisting of net operating loss carryforwards, stock-based compensation and unrealized gain/losses. Based upon the income projections in the majority of the foreign jurisdictions, the Company believes it will realize the benefit of its future deductible differences in these jurisdictions. As such, the Company has not recorded a valuation allowance against the net deferred tax assets in these foreign jurisdictions. Brazil, Germany and the Netherlands have generated deferred tax assets, which consist primarily of net operating loss carryforwards. The Company has concluded that it is more likely than not that it will not recognize the future benefits of the deferred tax assets in these jurisdictions, and accordingly, a full valuation allowance has been recorded against these foreign deferred tax assets.

As of December 31, 2024, the Company had federal and state net operating loss carryforwards of $134.9 million and $354.8 million, respectively, available to reduce future taxable income. Federal and state net operating loss carry forwards of $69.1 million and $341.0 million will expire at various dates between 2025 and 2041. Federal and state net operating loss carryforwards of $65.8 million and $13.8 million, respectively, can be carried forward indefinitely. Utilization of these net operating losses could be limited

under Section 382 of the Internal Revenue Code and similar state laws based on future ownership changes and the value of the Company's stock. Additionally, the Company has $40.9 million and $48.9 million of federal and state research and development credits, respectively, and $301.9 million of federal orphan drug tax credits available to offset future tax liabilities. These federal and state research and development credits expire between 2034 and 2044 and between 2031 and 2039, respectively. The federal orphan drug credits expire between 2034 and 2044. The Company also has foreign net operating loss carryforwards of $13.5 million, mainly derived from the net operating loss generated by its subsidiary in Brazil, which may be carried forward indefinitely.

The Company, or one of its subsidiaries, files income tax returns in the U.S., and various state and foreign jurisdictions. The federal, state and foreign income tax returns are generally subject to tax examinations for the tax years ended December 31, 2021 through December 31, 2024. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service, state or foreign tax authorities to the extent utilized in a future period.

The following table summarizes the reconciliation of the beginning and ending amount of total unrecognized tax benefits for each of the periods indicated:

	For the Year Ended December 31,		
	2024	2023	2022
		(in thousands)	
Balance at beginning of the period	$ 65,030	$ 61,704	$ 53,815
Increase related to current year tax positions	1,728	4,126	8,079
Increase related to prior year tax positions	178	—	—
Decrease related to prior year tax positions	(401)	(800)	(190)
Balance at end of the period	$ 66,535	$ 65,030	$ 61,704

The balance of total unrecognized tax benefits at December 31, 2024, if recognized, would not affect the effective tax rate on income from continuing operations, due to a full valuation allowance against the Company's U.S. deferred tax assets. The Company does not expect the amount of unrecognized tax benefits to change significantly in the next twelve months. The Company's policy is to recognize interest and/or penalties related to income tax matters in income tax expense. It had no accrual for interest or penalties on its consolidated balance sheets at December 31, 2024 or 2023. No interest and/or penalties were recognized in 2024, 2023, or 2022.

The Company's intent is to only make distributions from non-U.S. subsidiaries in the future when they can be made at no net tax cost. Otherwise, the Company considers all of its foreign earnings to be permanently reinvested outside of the U.S. and has no plans to repatriate these foreign earnings to the U.S. The Company has no material unremitted earnings from its non-U.S. subsidiaries.

19. LEASES

The Company has real estate operating leases in Cambridge, Andover, Burlington and Bedford, Massachusetts, Dublin and Columbus, Ohio, and Durham, North Carolina that provide for scheduled annual rent increases throughout each lease's term. The Company has also identified leases embedded in certain of its manufacturing and supply agreements as the Company determined that it controls the use of the facilities and related equipment therein. For more information related to the lease embedded in manufacturing and supply agreements with Catalent, Inc. ("Catalent"), please refer to *Note 22, Commitments and Contingencies*.

Bedford, Massachusetts

On April 22, 2022, the Company entered into a lease agreement (the "Bedford Lease") for 288,000 square feet of to-be-constructed research and development and manufacturing space in Bedford, Massachusetts. The term of the Bedford Lease commences upon the landlord's completion of the initial construction of the core and shell of the building, at which time the Company will obtain control of the premises and commence internal construction activities. The initial term of the Bedford Lease is anticipated to terminate on December 31, 2038. The Company has two options to extend the lease for a period of ten years each, exercisable under certain conditions and at a market rate determined in accordance with the lease agreement. The lease commenced in May 2023 as the Company obtained control of the premises.

The Bedford Lease provides for a tenant improvement allowance from the landlord of $72.0 million to be used towards costs incurred by the Company in the design and construction of the premises, which was recorded as a reduction to ROU assets and lease liabilities. In August 2024, the Company entered into an amendment to the Bedford Lease to extend the term in which the tenant

improvement allowance could be reimbursed from the landlord (the "Bedford Amendment") through December 2, 2025. The Company also agreed to reimburse the landlord for certain costs incurred in order to fund the extension of the reimbursement period associated with the tenant improvement allowance, totaling $2.9 million. The Bedford Amendment was accounted for as a modification to the Bedford Lease.

In May 2022, in connection with the execution of the Bedford Lease, the Company issued a letter of credit collateralized by cash deposits of approximately $8.4 million, which was included in other non-current assets of the Company's consolidated balance sheets. Such letter of credit shall be reduced to approximately $5.6 million at the commencement of the fourth rent year, provided certain conditions set forth in the Bedford Lease are satisfied. Additionally, the Company is responsible for reimbursing the landlord for the Company's share of the property's operating expenses and property taxes.

The Company had a lease liability and ROU asset of $143.3 million and $91.6 million, respectively, on the consolidated balance sheets as of December 31, 2024 related to the Bedford Lease. Tenant improvement costs incurred by the Company that had been reimbursed by the landlord totaled $40.3 million as of December 31, 2024 and are recorded as an increase to the lease liability within the Company's consolidated balance sheets.

Columbus, Ohio

In December 2018, the Company entered into a lease agreement for a research and development facility in Columbus, Ohio, which was subsequently amended in May 2022 (the "Columbus Amendment," together with the Columbus Amendment, the lease agreement is referred to as the "Columbus Lease"). The Columbus Lease expands from its current form of approximately 78,000 square feet to 167,000 square feet through a series of expansion spaces commencing at various periods through January 1, 2025.

Each expansion space commences on the date when the landlord will deliver control of that space for the Company to carry out design and construction activities (the "Columbus Commencement Date"). The Company is obligated to pay rent on each expansion space nine months after the Columbus Commencement Date. The Columbus Lease expires on December 31, 2036, and the Company has options to extend the lease by five years in both 2036 and 2041. Each option is exercisable under certain conditions and at a market rate determined in accordance with the lease agreement.

The Company commenced design and construction activities on areas of the premises of approximately 18,000 square feet (the "Second Expansion Space"), 36,000 square feet (the "Initial Expansion Space") and 19,000 square feet (the "Third Expansion Space") on June 1, 2022, October 1, 2022 and September 1, 2023, respectively. As a result, it was determined that the lease related to the Second Expansion Space, the Initial Expansion Space and the Third Expansion Space had commenced on those three dates, respectively. The total ROU asset and lease liability associated with the Columbus Lease, inclusive of the Third Expansion Space, the Second Expansion Space and the Initial Expansion Space, was $11.1 million and $19.3 million, respectively, as of December 31, 2024.

Lease Obligations

As of December 31, 2024, ROU assets for operating leases were $148.3 million and operating lease liabilities were $205.7 million. The following table contains a summary of the lease costs recognized and other information pertaining to the Company's operating leases for the periods indicated:

	For the Year Ended December 31,	
	2024	2023
	(in thousands)	
Lease cost		
Operating lease cost	$ 35,020	$ 29,895
Variable lease cost	68,624	38,442
Total lease cost	$ 103,644	$ 68,337
Other information		
Operating lease payments	$ 33,225	$ 21,608
Operating lease liabilities arising from obtaining ROU assets	$ 35,361	$ 80,203
Weighted-average remaining lease term	11.7 years	11.2 years
Weighted-average discount rate	8.0%	9.1%

The following table summarizes maturities of lease liabilities and the reconciliation of lease liabilities as of December 31, 2024:

	For the Year Ended December 31, 2024
	(in thousands)
2025	$ 31,758
2026	31,530
2027	31,556
2028	32,279
2029	25,796
Thereafter	229,353
Total minimum lease payments	382,272
Less: imputed interest and tenant incentive reimbursable by lessors	(176,526)
Total operating lease liabilities	$ 205,746
Included in the consolidated balance sheet:	
Current portion of lease liabilities within other current liabilities	$ 13,273
Lease liabilities, non-current	192,473
Total operating lease liabilities	$ 205,746

20. EARNINGS (LOSS) PER SHARE

Basic earnings (loss) per share is computed by dividing net income (loss) by the weighted-average number of shares of common stock outstanding. Diluted earnings per share is computed based on the treasury stock method for stock awards and the if-converted method for convertible debt by dividing net income by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding. Given that the Company recorded a net loss for the years ended December 31, 2023 and 2022, there is no difference between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive and are, therefore, excluded from the diluted net loss per share calculation.

The following table sets forth the computation of basic and diluted earnings (loss) per common share:

	For the Year Ended December 31,		
	2024	2023	2022
	(in thousands, except per share amounts)		
Numerator:			
Net income (loss) – basic	$ 235,239	$ (535,977)	$ (703,488)
Add: interest expense, net of tax, on the Company's convertible debt	17,000	—	—
Net income (loss) – diluted	$ 252,239	$ (535,977)	$ (703,488)
Denominator:			
Weighted-average common shares outstanding, basic	95,075	92,398	87,559
Effect of dilutive securities:			
Common stock issuable under the Company's equity incentive plans	3,513	—	—
Common stock issuable under the Company's convertible debt	9,287	—	—
Weighted-average common shares outstanding, diluted	107,875	92,398	87,559
Earnings (loss) per common share, basic	$ 2.47	$ (5.80)	$ (8.03)
Earnings (loss) per common share, diluted	$ 2.34	$ (5.80)	$ (8.03)

The following table summarizes potential shares of common stock that were excluded from the computation of diluted earnings per share as they were anti-dilutive:

	For the Year Ended December 31,		
	2024	2023	2022
		(in thousands)	
Common stock issuable under the Company's equity incentive plans	2,520 [1]	11,956 [2]	11,229 [3]
Common stock issuable under the Company's convertible debt	—	9,542	13,813
Total number of potentially issuable common stock	2,520	21,498	25,042

[1] As of December 31, 2024, the anti-dilutive common stock issuable under the Company's equity incentive plans excludes 1.2 million shares that are dilutive but have performance or market conditions that were not met as of the end of the period.

[2] As of December 31, 2023, the anti-dilutive common stock issuable under the Company's equity incentive plans includes 1.1 million shares that have performance or market conditions that were not met. These were anti-dilutive as the Company was in a net loss position at the end of the period.

[3] As of December 31, 2022, the anti-dilutive common stock issuable under the Company's equity incentive plans includes 1.1 million shares that have performance or market conditions that were not met. These were anti-dilutive as the Company was in a net loss position at the end of the period.

21. SEGMENT INFORMATION

The Company, together with its wholly-owned subsidiaries, is a commercial-stage biopharmaceutical company focused on helping patients through the discovery and development of unique RNA-targeted therapeutics, gene therapy and other genetic therapeutic modalities for the treatment of rare diseases. The Company’s research and development organization is responsible for the research and discovery of new product candidates and supports development and registration efforts for potential future products. The Company’s supply chain organization manages the development of the manufacturing processes, clinical trial supply and commercial product supply. The Company’s commercial organization is responsible for worldwide commercialization of EXONDYS 51, VYONDYS 53 and AMONDYS 45 and domestic commercialization of ELEVIDYS. The Company is supported by other back-office general and administration functions. Consistent with this decision-making process, the Company’s CEO uses consolidated, single-segment financial information for purposes of evaluating performance, forecasting future period financial results, allocating resources and setting incentive targets.

The Company operates in one segment: discovering, developing, manufacturing and delivering therapies to patients with rare diseases. The Company’s reportable segment derives its revenues from sales of its products, which include the PMO Products and ELEVIDYS, as well as through collaboration and other revenues primarily generated from its collaboration arrangement with Roche and other revenues related to commercial ELEVIDYS supply to Roche and royalty revenue from Roche. The Company’s CEO, as the CODM, manages and allocates resources to the operations of the Company on a total company basis by assessing the overall level of resources available and how to best deploy these resources across functions and in line with the Company's strategic goals. The Company’s accounting policies associated with segment information are described in *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* to the consolidated financial statements.

The measure of segment profit or loss that the CODM uses to allocate resources and assess performance is the Company’s consolidated net income (loss). The CODM uses consolidated net income (loss) to assess the segment’s overall profitability. The table below includes information about the Company’s segment, including significant segment expenses, and a reconciliation to net income (loss):

	For the Year Ended December 31,		
	2024	2023	2022
	(in thousands)		
Total revenues	$ 1,901,979	$ 1,243,336	$ 933,013
Segment expenses and other segment items			
Cost of sales (excluding amortization of in-licensed rights)	319,099	150,343	139,989
Compensation and other personnel expenses	335,830	319,080	256,382
Manufacturing expenses	329,011	345,826	466,111
Clinical trial expenses	163,565	187,289	135,838
Facility- and technology-related expenses (excluding depreciation and amortization)	106,281	88,559	71,700
Research and development- other (excluding non-cash items) (a)	108,597	118,727	117,171
Selling, general and administrative- other (excluding non-cash items) (b)	226,598	181,310	124,948
Roche collaboration reimbursement	(127,107)	(106,885)	(117,807)
Other segment items (c)	(30,449)	(49,129)	(11,014)
Interest expense	18,391	22,010	53,248
Interest income	(30,635)	(36,257)	(16,488)
Income tax expense	25,535	15,879	13,525
Depreciation and amortization expense	37,724	44,397	41,864
Stock-based compensation expense	184,300	182,514	233,018
Gain from sale of Priority Review Voucher	—	(102,000)	—
Impairment of strategic investments	—	30,321	2,575
Loss on debt extinguishment	—	387,329	125,441
Segment net income (loss)	$ 235,239	$ (535,977)	$ (703,488)
Reconciliation of profit or loss			
Adjustments and reconciling items	—	—	—
Consolidated net income (loss)	$ 235,239	$ (535,977)	$ (703,488)

(a) Research and development-other includes professional services, up-front, milestone, and other expenses, pre-clinical expenses and research and other expenses.
(b) Selling, general and administrative-other includes professional services and other expenses.
(c) Other segment items included in segment net income (loss) include accretion of investment discount, net, change in fair value of derivatives and other, net, as well as the items separately presented and not defined as significant expenses below.

Significant expense categories that are regularly provided to the CODM include cost of sales (excluding amortization of in-licensed rights), compensation and other personnel expenses, manufacturing expenses, clinical trial expenses, facility- and technology-related expenses, research and development- other and selling, general and administrative- other. The other expense or income information are other segment items and include separate presentation of interest expense, income tax expense, depreciation and amortization, stock-based compensation, which are included in the measure of segment income (loss) but are not significant segment expenses.

Assets provided to the CODM for the single segment are consistent with those reported on the consolidated balance sheets.

22. COMMITMENTS AND CONTINGENCIES

Manufacturing Obligations

The Company has entered into long-term contractual arrangements from time to time for the provision of goods and services. The following table presents non-cancelable contractual obligations arising from long-term contractual arrangements, including obligations related to leases embedded in certain supply agreements:

	As of December 31, 2024
	(in thousands)
2025	$ 943,067
2026	141,233
2027	79,860
2028	72,341
Total manufacturing commitments*	$ 1,236,501

* Total manufacturing commitments include the Catalent Agreement (defined below), for which the Company determined has a lease embedded in it and, as such, has ROU assets and lease liabilities recorded on the consolidated balance sheets as of December 31, 2024.

Thermo Fisher Scientific, Inc.

The Company entered into a development, commercial manufacturing, and supply agreement as related to the Company's adherent manufacturing process for its gene therapy programs in June 2018 and, subsequently, entered into the first, second and third amendments in May 2019, July 2020 and October 2021, respectively, with Brammer Bio MA, LLC, an affiliate of Thermo Fisher Scientific, Inc. (“Thermo”) (collectively, the “Thermo Agreement”).

In October 2021, the Company executed a third amendment (the “Third Amendment”) that modified the terms of the Thermo Agreements, which significantly decreased the Company’s right of use of the facility’s capacity and reduced the fixed and in-substance fixed payments due over the remaining term of the agreement. Under the Third Amendment, the Company has committed to guaranteed purchases under the Third Amendment on a take-or-pay basis regardless of whether services or goods are ordered. During the year ended December 31, 2022, the Company did not satisfy the total annual guaranteed purchase requirements and, as a result, recognized a loss of approximately $54.0 million, which was classified as research and development expense in the accompanying consolidated statement of comprehensive income (loss) for the year ended December 31, 2022, with the outstanding liability reflected as accrued contract manufacturing costs within accrued expenses in the consolidated balance sheets as of December 31, 2022. No similar losses were incurred during the years ended December 31, 2023 or 2024.

In March 2023, the Company executed a fourth amendment (the “Fourth Amendment”) that modified the terms of the Thermo Agreement. The Fourth Amendment removed the previous minimum batch purchase commitment and associated fee for the remaining term of the Thermo Agreement, and instead implemented a fee of up to $60.0 million, to be paid in three installments of $20.0 million each by March 1, 2024, December 31, 2024 and December 31, 2025, respectively. During the year ended December 31, 2024, the Company paid the first $20.0 million installment due March 1, 2024, considered a nonrefundable advance payment.

On July 18, 2024, the Company issued a termination notice to Thermo to terminate the Thermo Agreement. The termination was effective as of August 21, 2024. The aggregate net impact of the termination was $55.4 million of research and development expense in the accompanying consolidated statements of comprehensive income (loss). As the Company had yet to obtain regulatory approval to produce commercial supply of ELEVIDYS at Thermo manufacturing facilities, the Company recorded the charges incurred as research and development expenses during the year ended December 31, 2024. Included in the net impact noted above are non-cash charges due to the termination of $62.7 million, related to the remaining unamortized nonrefundable advance payments. Additionally, there were unbilled service and material costs ordered by the Company as of the termination date of $29.2 million that were expensed as research and development expense during the year ended December 31, 2024. As related to the termination, costs reimbursable by Roche under the Roche Agreement and reflected as a reduction to research and development expenses were $36.5 million.

Catalent, Inc.

The Company entered into a manufacturing collaboration agreement and, subsequently, a manufacturing and supply agreement with Catalent, formerly Paragon Biosciences, Inc. in October 2018 and February 2019, respectively (collectively, the “Catalent Agreements”). Pursuant to the terms of the Catalent Agreements, Catalent agreed to provide the Company with two dedicated clean room suites and an option to reserve two additional clean room suites for its gene therapy programs, subject to certain minimum and maximum volume limitations. In September 2019, the Company exercised the option to gain access to the two additional clean room suites. The Company determined that the Catalent Agreements contained a lease because the Company had the right to direct the use of the facility and related equipment therein. The lease on all four dedicated clean room suites at Catalent commenced during 2020, which is when the dedicated clean room suites became available for use by the Company. In March 2021, the Company modified the terms of the Catalent Agreements, which decreased the Company’s right of use of certain dedicated clean room suites. As of December 31, 2024, the Company controls two clean room suites.

In November 2022, the Company modified certain terms of the Catalent Agreements which extended the term of the agreement through December 31, 2028, which represented a modification of the existing embedded lease over certain clean room suites. The modification resulted in the recognition of additional ROU assets and lease liabilities of $19.2 million, as well as reclassification of $3.9 million between long-term and short-term manufacturing deposits. The modification also removed certain fixed payments due over the remaining term of the agreement. Further, in order to maintain the Company's dedicated clean room suites, it has committed to guaranteed purchases under the Amendment on a take-or-pay basis regardless of whether services or goods are ordered. The Company has the ability to terminate the Catalent Agreements prior to expiration, subject to the payment of additional financial consideration. As of December 31, 2024, the Company believes it is probable that the guaranteed purchase requirements will be met in the normal course of business throughout the term of the Catalent Agreements.

Aldevron, LLC

The Company entered into a clinical and commercial supply agreement in December 2018, as subsequently amended in June 2020, with Aldevron LLC ("Aldevron") for the supply of plasmid DNA to fulfill its needs for gene therapy clinical trials and commercial supply (collectively, the "Aldevron Agreements"). Pursuant to the terms of the Aldevron Agreements, Aldevron agreed to reserve a certain amount of manufacturing capacity on a quarterly basis. In return, the Company is required to make advance payments to Aldevron related to the manufacturing capacity. The term of the Aldevron Agreements will expire on December 31, 2026. The Company has the option to extend the term of the Aldevron Agreements by one year if the Company delivers a written notice of its intention to extend to Aldevron no later than June 1, 2025.

In January 2025, the Company modified certain terms of the Aldevron Agreements which extended the term of the agreement through December 31, 2028 (the "Aldevron Amendment"). As a result of the Aldevron Amendment, the Company has the option to extend the term of the Aldevron Agreements by one year if the Company delivers a written notice of its intention to extend to Aldevron no later than June 1, 2028. Both parties have the right to early terminate without additional penalty. The Company has determined that the Aldevron Amendment does not contain an embedded lease because it does not convey the right to control the use of Aldevron's facility or related equipment therein.

Other Funding Commitments

The Company has several on-going clinical trials in various clinical trial stages. Its most significant clinical trial expenditures are to contract research organizations ("CROs"). The CRO contracts are generally cancellable at the Company's option. As of December 31, 2024, the Company had approximately $594.5 million in cancellable future commitments based on existing CRO contracts. For the years ended December 31, 2024, 2023 and 2022, the Company recognized approximately $110.1 million, $112.2 million and $78.7 million, respectively, for expenditures incurred by CROs.

Litigation

In the normal course of business, the Company from time to time is named as a party to various legal claims, actions and complaints, which have included and may include matters involving securities, employment, intellectual property, arising from the use of therapeutics utilizing its technology, or others. The Company records a loss contingency reserve for a legal proceeding when it considers the potential loss probable and it can reasonably estimate the amount of the loss or determine a probable range of loss. The Company provides disclosure when it considers a loss reasonably possible or when it determines that a loss in excess of a reserve is reasonably possible. The Company provides an estimate of such reasonably possible losses or an aggregate range of such reasonably possible losses, unless the Company believes that such an estimate cannot be made. The Company has not recorded any material accruals for loss contingencies, and in management's opinion, no material range of loss is estimable for the matters described below as of December 31, 2024.

On September 15, 2020, REGENXBIO INC. ("Regenx") and the Trustees of the University of Pennsylvania ("U-Penn") filed a lawsuit against the Company and Sarepta Therapeutics Three, LLC, in the U.S. District Court for the District of Delaware. The plaintiffs assert patent infringement of U.S. Patent No. 10,526,617 ("the '617 Patent") under 35 U.S.C.§§ 271(a)-(c) based on Sarepta's alleged direct or indirect manufacture and use of the patented cultured host cell technology allegedly used to make AAV gene therapy products, including SRP-9001 (approved June 22, 2023 in the U.S. as ELEVIDYS®). Specifically, the Complaint essentially includes the allegation that Sarepta's use, and the use by its contract manufacturers on its behalf, of a host cell containing a recombinant acid molecule that encodes a capsid protein having at least 95% amino acid identity to AAVrh10 infringes the '617 Patent asserted by Regenx. Plaintiffs seek injunctive relief, a judgment of infringement and willful infringement, damages that are no less than a reasonable royalty (treble damages), attorneys' fees and costs, and such other relief as the court deems just and proper. On January 5, 2024, the Court granted Sarepta's motion for summary judgment on the grounds that the asserted claims of Regenx's '617 Patent are invalid because they cover patent ineligible subject matter under 35 U.S.C. § 101. On January 12, 2024, the Court entered judgment and closed the case. Plaintiffs have appealed to the U.S. Court of Appeals for the Federal Circuit.

On June 20, 2023, Regenx and U-Penn commenced a second patent infringement lawsuit against Sarepta and its contract manufacturer, Catalent asserting patent alleged infringement of U. S. Patent No. 11,680,274 ("the '274 Patent"). In the second lawsuit, Regenx and U-Penn allege that Sarepta and Catalent's manufacture, use and commercial launch of ELEVIDYS® (formerly/also known as SRP-9001) infringe the '274 Patent. Sarepta answered the complaint on August 10, 2023, and a case schedule has been set

with a trial commencing on November 17, 2025. On February 21, 2024, Sarepta submitted a petition for Inter Partes Review for filing with the Patent Trial and Appeal Board ("PTAB") at the U.S. Patent and Trademark Office ("USPTO"). The petition seeks to invalidate the '274 Patent. On August 22, 2024, the PTAB instituted *inter partes* review of all challenged claims of the '274 Patent on all asserted grounds.

On July 13, 2021, Nippon Shinyaku Co., Ltd. ("Nippon Shinyaku" or "NS") filed a lawsuit against the Company in the U.S. District Court for the District of Delaware. NS asserted a claim for breach of contract arising from Sarepta filing seven petitions for Inter Partes Review ("IPR Petitions") with the PTAB at the USPTO in which Sarepta sought to invalidate certain NS patents concerning exon 53 skipping technology (U.S. Patent Nos. 9,708,361, 10,385,092, 10,407,461, 10,487,106, 10,647,741, 10,662,217, and 10,683,322, respectively, and collectively the "NS Patents"). In addition, NS asserted claims for patent infringement and willful infringement of each of the NS Patents allegedly arising from Sarepta's activities, including the sale of, its exon 53 skipping product, VYONDYS 53 (golodirsen). NS further sought a determination of non-infringement by NS alleged to arise from NS's activities, including the sale of, its exon 53 skipping product, Viltepso (viltolarsen) and invalidity of certain patents licensed to the Company from UWA (U.S. Patent Nos. 9,994,851, 10,227,590, and 10,266,827, collectively the "UWA Patents"). In its complaint, NS sought legal fees and costs, an unspecified amount of monetary relief (treble damages) attributed to Sarepta's alleged infringement, and such other relief as the court deems just and proper. In January 2022, the PTAB granted institution of all claims of all NS Patents in response to Sarepta's IPR Petitions and determined that Sarepta demonstrated a reasonable likelihood of success in proving that the NS Patents are unpatentable. NS filed a motion for preliminary injunction solely seeking Sarepta's withdrawal of the IPR Petitions, which was ultimately granted after the U.S. Court of Appeals for the Federal Circuit reversed and remanded to the district court on February 8, 2022. Sarepta subsequently withdrew the IPRs, which were terminated on June 14, 2022. On December 27, 2021, the district court partially granted and denied the motion to dismiss by Sarepta and ordered NS to file a Second Amended Complaint ("SAC"), which it did on January 14, 2022. In the SAC, NS maintained all claims of the original complaint of July 13, 2021, except a determination of non-infringement of the UWA Patents. On January 28, 2022, Sarepta filed its answer to the SAC, with defenses and counterclaims against NS and NS Pharma Inc. that include infringement of the UWA Patents allegedly arising from their activities concerning, including the sale of, its exon 53 skipping product, Viltepso (viltolarsen) and breach of contract. Sarepta also sought a determination of invalidity of the NS Patents. In its counterclaim complaint, Sarepta sought an award of relief in its defenses to NS' allegations, a judgment of breach of contract, a determination of invalidity of the NS Patents, a judgment of infringement and willful infringement of the UWA Patents, legal fees and costs, an unspecified amount of monetary relief (treble damages) attributable to NS' alleged infringement, and such other relief as the court deems just and proper. UWA has since been joined as a Plaintiff in Sarepta's counterclaims against NS. On August 14, 2023, the Court granted cross motions to amend the pleadings, allowing Sarepta to add a counterclaim against NS for inequitable conduct, and NS to add counterclaims against Sarepta for inequitable conduct and Walker Process fraud. The parties have since stipulated to the dismissal of NS's claim of infringement of its '361 Patent and certain claims of the '322 Patent, and NS's breach of contract claim. The Court bifurcated the Walker Process fraud claim on April 18, 2024, and granted Sarepta's motion for summary judgement of infringement of the '851 Patent and NS's motion for partial summary judgment of infringement of certain NS patents on May 1, 2024. After a jury trial in December 2024, the jury found that NS's '092 Patent is invalid as obvious and Sarepta's and UWA's '851 Patent is not invalid. The jury did not find that NS's infringement was willful. The jury awarded Sarepta approximately $115.2 million in damages for NS's infringement relating to its sales of Viltepso in the United States, and the parties stipulated to approximately $0.8 million in reasonable royalty damages for NS's sales of Viltepso outside of the United States, both through December 15, 2024. Judgement was entered on January 7, 2025. On February 14, 2025, Sarepta filed a motion seeking supplemental damages and NS filed post-trial motions challenging the jury's verdict, as well as briefing relating to its inequitable conduct claim, which was tried to the court in December 2024.

On or about June 5, 2023, Sarepta initiated a patent infringement lawsuit against Nippon Shinyaku in Japan, alleging that NS's production, sales and offers to sell Viltepso infringe Sarepta's Japanese Patent No. 6406782. NS filed its preliminary answer on July 13, 2023. A technical presentation session occurred on July 26, 2024. Sarepta subsequently abandoned its claims and the case was terminated on January 30, 2025.

On July 26, 2024, Genzyme Corporation filed a lawsuit against Sarepta Therapeutics, Inc. and Sarepta Therapeutics Three, LLC, in the U.S. District Court for the District of Delaware. The complaint asserts infringement of United States Patent Nos. 9,051,542 (the "'542 Patent") and 7,704,721 (the "'721 Patent") arising from Sarepta's alleged manufacture and sale of ELEVIDYS® (delandistrogene moxeparvovec-rokl). In its complaint, Genzyme seeks, inter alia, damages for the alleged infringement, including increased damages up to three times the amount found or assessed, together with prejudgment and post-judgment interest and costs. Following a partial motion to dismiss by Sarepta, Genzyme filed its First Amended Complaint on November 21, 2024. In its First Amended Complaint, Genzyme no longer alleges willfulness or indirect infringement before the filing of the complaint. Sarepta answered the First Amended Complaint on December 12, 2024. The Court entered a scheduling order, with a trial scheduled for January 25, 2027.

On December 20, 2024, Brammer Bio MA, LLC filed an arbitration demand against Sarepta relating to Sarepta's termination of the Thermo Agreement. Brammer Bio MA, LLC alleges claims for breach of contract, breach of the implied covenant of good faith

and fair dealing and a violation of MGL c. 93A, and seeks relief including damages, treble damages, and attorneys' fees and costs. On January 24, 2025, Sarepta filed its answer and asserted counterclaims for declaratory judgment and breach of contract, seeking relief including damages and attorneys' fees and costs.

23. SUBSEQUENT EVENT

On February 13, 2025 (the "Closing Date"), the Company entered into a credit agreement (the "Credit Agreement") with JPMorgan Chase Bank, N.A., as administrative agent (the "Administrative Agent") and as collateral agent and the lenders party thereto. The Credit Agreement provides for a five-year, $600.0 million senior secured revolving credit facility (the "Revolving Credit Facility").

Interest rates under the Revolving Credit Facility are variable and equal to the Secured Overnight Financing Rate plus a credit spread adjustment of 0.10% per annum ("Adjusted SOFR"), plus a margin of 1.125% to 1.75% per annum, or, at the Company's option, at a base reference rate equal to the highest of (a) the federal funds rate plus 0.50%, (b) the rate of interest last quoted by the Administrative Agent as its "base rate" and (c) the one-month Adjusted SOFR rate plus 1.00%, plus a margin of 0.125% to 0.75% per annum. The Company also will pay an unused commitment fee ranging from 0.20% to 0.35% per annum on the unused commitments.

The Credit Agreement contains customary representations and warranties, affirmative covenants, negative covenants and events of default. The Credit Agreement also contains financial covenants that are assessed on the last day of each of the Company's fiscal quarters, including certain financial ratios.

The Company paid $3.2 million in arrangement and up-front fees on the Closing Date. The Company may voluntarily prepay the outstanding revolving loans under the Revolving Credit Facility in whole or in part without premium or penalty provided that the prepayment shall be in certain amounts as specified therein. The Company is currently evaluating the Revolving Credit Facility to determine the impact on its consolidated financial statements and related disclosures. As of the date of this filing, the Company has not drawn upon the Revolving Credit Facility.

Exhibit 10.67

[***] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED.

EXCLUSIVE LICENSE AND COLLABORATION AGREEMENT

BY AND BETWEEN

ARROWHEAD PHARMACEUTICALS, INC.

AND

SAREPTA THERAPEUTICS, INC.

NOVEMBER 25, 2024

[***] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED.

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EXCLUSIVE LICENSE AND COLLABORATION AGREEMENT

THIS EXCLUSIVE LICENSE AND COLLABORATION AGREEMENT (this "**Agreement**"), entered into as of November 25, 2024 (the "**Execution Date**"), is entered into by and between Sarepta Therapeutics, Inc., a Delaware corporation having its principal offices at 215 First Street, Cambridge, MA 02142 ("**Sarepta**"), and Arrowhead Pharmaceuticals, Inc., a Delaware corporation having its principal offices at 177 East Colorado Boulevard, Suite 700, Pasadena, CA ("**Arrowhead**"). Arrowhead and Sarepta are referred to in this Agreement individually as a "**Party**" and collectively as the "**Parties**."

RECITALS

WHEREAS, Sarepta is a biotechnology company engaged in the research, development, and commercialization of products useful in the amelioration, treatment, or prevention of genetic human diseases and conditions;

WHEREAS, Arrowhead is a biopharmaceutical company focused on developing medicines that treat intractable diseases by silencing the genes that cause them, including advancing RNA interference based treatments for protein-based genetic disorders;

WHEREAS, the Parties wish to enter into a collaboration to develop targeted siRNA therapies against (a) DUX4 for the treatment of facioscapulohumeral muscular dystrophy, (b) DMPK for the treatment of type 1 myotonic dystrophy, (c) ATXN1, ATXN2, and ATXN3 for the treatment of Ataxias, (d) MMP7 for the treatment of idiopathic pulmonary fibrosis, (e) HTT for the treatment of Huntington's Disease, and (f) other Collaboration Targets targeting skeletal muscle, the central nervous system, or cardiomyocytes; and

WHEREAS, Sarepta wishes to obtain, and Arrowhead desires to grant, an exclusive worldwide license under certain Patent Rights, Know-How, and other intellectual property rights Controlled by Arrowhead to Research, Develop, Manufacture, Commercialize, and otherwise Exploit the Licensed Compounds and Licensed Products on the terms and conditions set forth herein.

NOW, **THEREFORE**, the Parties hereby agree as follows:

1. DEFINITIONS

Unless specifically set forth to the contrary herein, the following terms, whether used in the singular or plural, will have the respective meanings set forth below:

1.1 "**Acquired Business**" has the meaning set forth in Section 2.10.3 (Acquired Business Exception).

1.2 "**Acquirer**" means, collectively, the Third Party referenced in the definition of Change of Control and such Third Party's Affiliates, other than the applicable Party in the definition of Change of Control and such Party's Affiliates immediately prior to the closing of such Change of Control.

1.3 "**Additional R&D Activities**" has the meaning set forth in Section 3.1.4 (Additional R&D Responsibilities).

1.4 "**Additional R&D Budget**" has the meaning set forth in Section 3.1.4 (Additional R&D Responsibilities).

1.5 "**Additional R&D Plan**" has the meaning set forth in Section 3.1.4 (Additional R&D Responsibilities).

1.6 "**Adverse Event**" means any untoward medical occurrence in a human clinical study subject or in a patient who is administered a Licensed Product, whether or not considered related to such Licensed Product, including any undesirable sign (including abnormal laboratory findings of clinical concern), symptom, or disease associated with the use of a Licensed Product.

1.7 "**Affiliate**" means any Person directly or indirectly controlled by, controlling, or under common control with, a Party, but only for so long as such control continues. For purposes of this definition, "**control**" (including, with correlative meanings, "**controlled by**," "**controlling**," and "**under common control with**") will be presumed to exist with respect to a Person in the event of the possession, direct or indirect, of (a) the power to direct or cause the direction of the management and policies of such Person (whether through ownership of securities, by contract or otherwise), or (b) 50% or more of the voting securities or other comparable equity interests. The Parties acknowledge that in the case of certain entities organized under the laws of certain countries outside of the United States, the maximum percentage ownership permitted by law for a foreign investor may be less than 50%, and that in such case, such lower percentage will be substituted in the preceding sentence, *provided* that such foreign investor has the power to direct or cause the direction of the management and policies of such Person. Neither of the Parties will be deemed to be an "**Affiliate**" of the other solely as a result of their entering into this Agreement. The Parties acknowledge that for the purposes of this Agreement, Visirna Therapeutics, Inc. will not be an Affiliate of Arrowhead.

1.8 "**Agreement**" has the meaning set forth in the preamble.

1.9 "**Alliance Manager**" has the meaning set forth in Section 7.1 (Alliance Managers).

1.10 "**Annual Fees**" has the meaning set forth in Section 8.2 (Annual Fees).

1.11 "**Annual Net Sales**" means the aggregate Net Sales resulting from the sale of all Licensed Products Directed To a given Target in the Territory by Sarepta, its Affiliates, or its or their Sublicensees during a given Calendar Year.

1.12 "**Antitrust Clearance Date**" means the earliest date on which all applicable waiting periods and approvals required under Antitrust Laws with respect to the transactions contemplated under this Agreement have expired or have been terminated (in the case of waiting periods) or been received (in the case of approvals), in each case, without the imposition of any conditions.

1.13 "**Antitrust Filing**" means any filing with the United States Federal Trade Commission and the Antitrust Division of the United States Department of Justice and any other applicable Governmental Authority in the Territory, as required under any Antitrust Laws with respect to the transactions contemplated under this Agreement, together with all required documentary attachments thereto.

1.14 "**Antitrust Laws**" means any federal, state, or foreign law, regulation, or decree, including the HSR Act, designed to prohibit, restrict, or regulate actions for the purpose or effect of monopolization or restraint of trade.

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1.15 "**Approved Sarepta CMO**" has the meaning set forth in Section 5.4 (Manufacturing Technology Transfer).

1.16 "**Arbitrator**" has the meaning set forth in Section 15.3.1 (Expedited Arbitration).

1.17 "**Arising Delivery Ligand Know-How**" has the meaning set forth in Section 12.1.2(a) (Arrowhead).

1.18 "**Arising Delivery Ligand Patent Rights**" has the meaning set forth in Section 12.1.2(a) (Arrowhead).

1.19 "**Arising Know-How**" means any and all Know-How conceived, invented, developed, or otherwise made during the Term by or on behalf of one or more Personnel of a Party (or any of its Affiliates, licensees, sublicensees, or subcontractors), either alone or jointly with one or more Personnel of the other Party (or any of its Affiliates, licensees, sublicensees, or subcontractors), in each case, in the performance of activities relating to the Exploitation of Licensed Compounds or Licensed Products under this Agreement.

1.20 "**Arising Patent Rights**" means any Patent Right that (a) has a priority date after the Effective Date, and (b) Covers any Arising Know-How.

1.21 "**ARO-ATXN1**" means the to-be-selected chemical composition that is the CTA-ready candidate internally coded by Arrowhead as ARO-ATXN1.

1.22 "**ARO-ATXN2**" means the chemical composition internally coded by Arrowhead as ARO-ATXN2, the chemical structure of which is set forth on **Schedule 1.22** (ARO-ATXN2 Structure).

1.23 "**ARO-ATXN3**" means the to-be-selected chemical composition that is the CTA-ready candidate internally coded by Arrowhead as ARO-ATXN3.

1.24 "**ARO-DM1**" means the chemical composition internally coded by Arrowhead as ARO-DM1, the chemical structure of which is set forth on **Schedule 1.24** (ARO-DM1 Structure).

1.25 "**[***]**" means [***].

1.26 "**[***]**" has the meaning set forth in [***].

1.27 "**ARO-DUX4**" means the chemical composition internally coded by Arrowhead as ARO-DUX4, the chemical structure of which is set forth on **Schedule 1.27** (ARO-DUX4 Structure).

1.28 "**ARO-HTT**" means the chemical composition that is the CTA-ready candidate internally coded by Arrowhead as ARO-HTT, the chemical structure of which is set forth on **Schedule 1.28** (ARO-HTT Structure).

1.29 "**ARO-MMP7**" means the chemical composition internally coded by Arrowhead as ARO-MMP7, the chemical structure of which is set forth on **Schedule 1.29** (ARO-MMP7 Structure).

1.30 "**Arrowhead**" has the meaning set forth in the preamble.

1.31 "**Arrowhead Arising Know-How**" has the meaning set forth in Section 12.1.2(a) (Arrowhead).

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1.32 "**Arrowhead Arising Patent Right**" has the meaning set forth in Section 12.1.2(a) (Arrowhead).

1.33 "**Arrowhead BBB Platform**" means the Know-How or other intellectual property rights Controlled by Arrowhead or its Affiliates that is related to, or Patent Rights Controlled by Arrowhead or its Affiliates that Cover, any Arrowhead Platform that utilizes the conjugation or other incorporation (or fragments thereof) of an antibody that is designed to shuttle certain compounds, including RNAi Molecules, across the blood brain barrier, and includes the platform disclosed in the Patent Rights listed in **Schedule 1.33** (Arrowhead BBB Platform Patent Rights).

1.34 "**Arrowhead Cardiomyocyte Platform**" means the Know-How or other intellectual property rights Controlled by Arrowhead or its Affiliates that is related to, or Patent Rights Controlled by Arrowhead or its Affiliates that Cover, any Arrowhead Platform that utilizes the conjugation or other incorporation of a ligand that is designed to deliver compounds, including RNAi Molecules, to cardiomyocytes, and includes the platform disclosed in the Patent Rights listed in **Schedule 1.34** (Arrowhead Cardiomyocyte Platform Patent Rights).

1.35 "**Arrowhead Development Costs**" has the meaning set forth in Section 3.4.1 (Arrowhead Development Costs Reimbursement).

1.36 "**Arrowhead Excluded Know-How**" means, collectively, any and all Know-How (a) relating to Arrowhead's RNAi Molecule trigger sequence selection and design process, or (b) that Arrowhead or any of its Affiliates comes to own or otherwise Control after the Execution Date relating to the Manufacture of RNAi Molecules generally, but only to the extent such Know-How is not (i) utilized in connection with any Development or Manufacturing work performed by Arrowhead or any of its Affiliates either (A) prior to the Execution Date for itself or (B) for Sarepta under this Agreement during the Term or under any other supply or development agreement or plan between the Parties after the Effective Date, (ii) otherwise disclosed in writing by Arrowhead to Sarepta during the Term, (iii) necessary for the Exploitation of a Licensed Compound or Licensed Product, or (iv) Arising Delivery Ligand Know-How.

1.37 "**Arrowhead Excluded Patent Rights**" means any Patent Rights that Cover any Arrowhead Excluded Know-How.

1.38 "**Arrowhead Indemnitees**" has the meaning set forth in Section 11.2 (Indemnification by Sarepta).

1.39 "**Arrowhead Know-How**" means any and all Know-How that (a) relates to the composition of matter, formulation, form, or a method of use or treatment, delivery, or Manufacture of a Licensed Compound or a Licensed Product, (b) is Controlled by Arrowhead or any of its Affiliates as of the Effective Date or during the Term, and (c) is necessary or reasonably useful to Exploit one or more Licensed Compounds or Licensed Products in the Field in the Territory, *including* any and all Arrowhead Arising Know-How and Arrowhead's interest in any and all Joint Arising Know-How but *excluding* Arrowhead Excluded Know-How. Notwithstanding anything herein to the contrary, Arrowhead Know-How excludes the Licensed Product-Specific Patent Rights and the Arrowhead Platform Patent Rights.

1.40 "**Arrowhead Manufacturing Know-How**" has the meaning set forth in Section 5.4 (Manufacturing Technology Transfer).

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1.41 "**Arrowhead Patent Rights**" means any and all Patent Rights that (a) are Controlled by Arrowhead or any of its Affiliates as of the Effective Date or during the Term and (b) (i) Cover a Licensed Compound or a Licensed Product (including, for clarity, its composition of matter, formulation, form, or a method of use or treatment, delivery, or Manufacture) or (ii) are necessary or reasonably useful to Exploit one or more Licensed Compounds or Licensed Products in the Field in the Territory, *including* any and all Arrowhead Arising Patent Rights and Arrowhead's interest in any and all Joint Arising Patent Rights but *excluding* all Arrowhead Excluded Patents Rights. The Arrowhead Patent Rights include the Arrowhead Platform Patent Rights.

1.42 "**Arrowhead Platform**" means Arrowhead's proprietary siRNA platform for RNAi Molecule sequence selection and delivery, including for Licensed Products and Licensed Compounds (*i.e.*, TRiM™ technology). For clarity, the Arrowhead Platform includes the Arrowhead BBB Platform, Arrowhead Cardiomyocyte Platform, and Arrowhead SM Platform.

1.43 "**Arrowhead Platform Patent Rights**" means any and all Arrowhead Patent Rights that are not Licensed Product-Specific Patent Rights. The Arrowhead Platform Patent Rights relevant to the contemplated Licensed Compounds and Licensed Products as of the Execution Date, and, if applicable, the Effective Date, are set forth on **Schedule 1.43** (Arrowhead Platform Patent Rights).

1.44 "**Arrowhead Prosecuted Patent Rights**" has the meaning set forth in Section 12.2.2(a) (Arrowhead's Right to Prosecute Patents).

1.45 "**Arrowhead Records**" has the meaning set forth in Section 8.7.3 (Records and Audits).

1.46 "**[***]**" means [***].

1.47 "**Arrowhead SM Platform**" means the Know-How or other intellectual property rights Controlled by Arrowhead or its Affiliates that is related to, or Patent Rights Controlled by Arrowhead or its Affiliates that Cover, any Arrowhead Platform that utilizes the conjugation or other incorporation of a ligand that is designed to deliver compounds, including RNAi Molecules, to skeletal muscle tissue, and includes the platform disclosed in the Patent Rights listed in **Schedule 1.47** (Arrowhead SM Platform Patent Rights).

1.48 "**Arrowhead Technology**" means, collectively, (a) the Arrowhead Patent Rights and (b) the Arrowhead Know-How.

1.49 "**Assigned Regulatory Submissions**" means all INDs, MAAs, and other Regulatory Approvals or Regulatory Submissions assigned by Arrowhead to Sarepta pursuant to Section 4.2 (Assignment of Regulatory Submissions).

1.50 "**Assumed C1 Program Development Activities**" has the meaning set forth in Section 3.1.1(a) (Arrowhead Category 1 Program Development Responsibility).

1.51 "**ATXN1**" means ataxin-1.

1.52 "**ATXN1 Backup Compound**" means any RNAi Molecule Directed To ATXN1 that is Controlled by Arrowhead or any of its Affiliates as of the Execution Date or, subject to Section 2.10 (Exclusivity), during the Term, excluding ARO-ATXN1.

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1.53 "**ATXN1 Program**" means the program for the Research, Development, Manufacture, Commercialization, and other Exploitation of Licensed ATXN1 Compounds and Licensed ATXN1 Products.

1.54 "**ATXN2**" means ataxin-2.

1.55 "**ATXN2 Backup Compound**" means any RNAi Molecule Directed To ATXN2 that is Controlled by Arrowhead or any of its Affiliates as of the Execution Date or, subject to Section 2.10 (Exclusivity), during the Term, excluding ARO-ATXN2.

1.56 "**ATXN2 Phase I Clinical Trial**" means the Phase I Clinical Trial titled "A Phase 1 Placebo-Controlled Dose Escalating Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of ARO-ATXN2 in Adult Subjects with Spinocerebellar Ataxia Type 2" (Clinical Trial ID: NCT06672445).

1.57 "**ATXN2 Program**" means the program for the Research, Development, Manufacture, Commercialization, and other Exploitation of Licensed ATXN2 Compounds and Licensed ATXN2 Products.

1.58 "**ATXN3**" means ataxin-3.

1.59 "**ATXN3 Backup Compound**" means any RNAi Molecule Directed To ATXN3 that is Controlled by Arrowhead or any of its Affiliates as of the Execution Date or during the Term, excluding ARO-ATXN3.

1.60 "**ATXN3 Program**" means the program for the Research, Development, Manufacture, Commercialization, and other Exploitation of Licensed ATXN3 Compounds and Licensed ATXN3 Products.

1.61 "**Audited Party**" has the meaning set forth in Section 8.7.3 (Records and Audits).

1.62 "**Auditing Party**" has the meaning set forth in Section 8.7.3 (Records and Audits).

1.63 "**Auditor**" has the meaning set forth in Section 8.7.3 (Records and Audits).

1.64 "**Bankrupt Party**" has the meaning set forth in Section 13.4 (Termination for Bankruptcy).

1.65 "**Bankruptcy Code**" means Title 11, United States Code, as amended, or analogous provisions of Law outside the United States.

1.66 "**Breaching Party**" has the meaning set forth in Section 13.5.1 (Material Breach and Cure Period).

1.67 "**Business Day**" means a calendar day other than a Saturday, Sunday, or a bank or other public holiday in Boston, Massachusetts or Pasadena, California.

1.68 "**Calendar Quarter**" means the respective periods of three consecutive calendar months ending on March 31st, June 30th, September 30th, or December 31st in any Calendar Year; *provided*, *however*, that the first Calendar Quarter of the Term will extend from the Effective Date to the end of the first complete Calendar Quarter thereafter.

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1.69 "**Calendar Year**" means any calendar year beginning on January 1st and ending on December 31st, *provided*, *however*, that the first Calendar Year of the Term will begin on the Effective Date and end on December 31.

1.70 "**Cardiomyocyte Target**" means any genetic target that a potential therapeutic product could be Directed To that would [***] cardiomyocytes.

1.71 "**Category 1 Development Budget**" has the meaning set forth in Section 3.1.1(c)(i) (Category 1 Development Plans).

1.72 "**Category 1 Development Plan**" has the meaning set forth in Section 3.1.1(c)(i) (Category 1 Development Plans).

1.73 "**Category 1 Lead Compounds**" means ARO-DUX4, ARO-DM1, ARO-ATXN2, and ARO-MMP7, in each case, individually or collectively as the context requires.

1.74 "**Category 1 Programs**" means the ATXN2 Program, the DM1 Program, the DUX4 Program, and the MMP7 Program, in each case, individually or collectively as the context requires.

1.75 "**Category 2 CTA Ready Data**" has the meaning set forth in Section 3.1.2(b) (Category 2 Development Plans).

1.76 "**Category 2 CTA Ready Package**" has the meaning set forth in Section 3.1.2(b) (Category 2 Development Plans).

1.77 "**Category 2 Development Plan**" has the meaning set forth in Section 3.1.2(b) (Category 2 Development Plans).

1.78 "**Category 2 Program Research Activities**" has the meaning set forth in Section 3.1.2(b) (Category 2 Development Plans).

1.79 "**Category 2 Programs**" means the ATXN1 Program, the ATXN3 Program, and the HTT Program, in each case, individually or collectively as the context requires.

1.80 "**Category 3 CTA Ready Data**" has the meaning set forth in Section 3.1.3(d) (Category 3 Development Plans).

1.81 "**Category 3 CTA Ready Package**" has the meaning set forth in Section 3.1.3(d) (Category 3 Development Plans).

1.82 "**Category 3 Development Plan**" has the meaning set forth in Section 3.1.3(d) (Category 3 Development Plans).

1.83 "**Category 3 Program Research Activities**" has the meaning set forth in Section 3.1.3(d) (Category 3 Development Plans).

1.84 "**Category 3 Programs**" means the programs for the Research, Development, Manufacture, Commercialization, and other Exploitation of Licensed C3 Compounds and Licensed C3 Products, including the [***], in each case, individually or collectively as the context requires.

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1.85 "**Change of Control**" means, with respect to a Party, that: (a) any Third Party acquires directly or indirectly the beneficial ownership of any voting security of such Party, or if the percentage ownership of such Third Party in the voting securities of such Party is increased through stock redemption, cancellation, or other recapitalization, and immediately after such acquisition or increase such Third Party is, directly or indirectly, the beneficial owner of voting securities representing at least 50% of the total voting power of all of the then-outstanding voting securities of such Party; (b) a merger, consolidation, recapitalization, or reorganization of such Party is consummated, other than any such transaction that would result in shareholders or equity holders of such Party immediately prior to such transaction owning at least 50% of the outstanding voting securities of the surviving entity (or its parent entity) immediately following such transaction; (c) the shareholders or equity holders of such Party approve a plan of complete liquidation of such Party, or an agreement for the sale or disposition by such Party of all or substantially all of such Party's assets, other than pursuant to the transactions described above or to an Affiliate; or (d) the sale or transfer to a Third Party of all or substantially all of such Party's consolidated assets taken as a whole. Notwithstanding the foregoing, any transaction or series of transactions effected for the purpose of financing the operations of the applicable Party or one or more of its applicable Affiliates (such as an initial public offering or other offering of equity securities to non-strategic investors) will not be deemed a "**Change of Control**" for purposes of this Agreement.

1.86 "**Clinical Trial**" means any clinical investigation in which a pharmaceutical product is administered or dispensed to, or used involving human subjects, including any Phase I Clinical Trial, Phase II Clinical Trial, Phase III Clinical Trial, or any post-approval clinical trial in humans.

1.87 "**Clinical Trial Regulatory Submissions**" means all INDs and other Regulatory Submissions in the Territory related to any Clinical Trial, including the Ongoing Development Trials.

1.88 "**CMC Activities**" means, with respect to a Licensed Compound or Licensed Product, all Manufacturing activities (including the generation of all CMC Data) necessary to support the Development or Commercialization of such Licensed Compound or Licensed Product, as applicable, at the applicable stage of Development or Commercialization, including formulation, process development, process qualification and validation, scale-up, analytic development, product characterization, stability testing, quality assurance, and quality control.

1.89 "**CMC Data**" means the chemistry, manufacturing and controls data for each Licensed Compound or Licensed Product, as applicable, required by applicable Law to be included or referenced in, or that otherwise supports, an application for Regulatory Approval for such Licensed Product.

1.90 "**CMO**" means a contract manufacturing organization or a contract testing organization.

1.91 "**CNS Target**" means (a) [***], and (b) any other genetic target that a potential therapeutic product could be Directed To that would inhibit or otherwise modulate expression within the central nervous system, but excluding [***].

1.92 "**[***]**" has the meaning set forth in [***].

1.93 "**[***]**" has the meaning set forth in [***].

1.94 "**Collaboration Target**" means each of the targets accepted by Arrowhead pursuant to Section 3.1.3(a) (Selection of Collaboration Targets).

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1.95 "**Collaboration Term**" means, on a Collaboration Target-by-Collaboration Target basis, the earlier of (a) the CTA Ready Package Acceptance Date for such Collaboration Target, (b) the date on which the [***] has been achieved for such Collaboration Target, or (c) the [***] anniversary of the Effective Date, *provided* that, solely in the case of this clause (c), (i) if a Replacement Target for such Collaboration Target is selected by Sarepta as permitted under Section 3.1.3(b) (Sarepta Collaboration Target Substitution Right) and accepted as a new Collaboration Target by Arrowhead pursuant to Section 3.1.3(a) (Selection of Collaboration Targets) and (ii) the [***] anniversary of the Execution Date is less than [***] after the date on which the Parties agree on a Category 3 Development Plan for such new Collaboration Target pursuant to Section 3.1.3(d) (Category 3 Development Plans), then such [***] period will be extended by such an amount of time as is necessary such that there are [***] from the date on which Arrowhead accepts such Replacement Target as a new Collaboration Target.

1.96 "**Combination Product**" means a Licensed Product that contains or comprises a Licensed Compound as a therapeutically active pharmaceutical ingredient together with one or more other therapeutically active pharmaceutical ingredients other than a Licensed Compound (an "**Other Component**") that are (a) either coformulated or copackaged together and sold either as a fixed dose/unit or as separate doses/units in a single package, or otherwise are sold together for a single price, but excluding devices, drug delivery vehicles, adjuvants, solubilizers and excipients used with a Licensed Compound, and not specifically related to an Other Component, or (b) defined as a "combination product" by the FDA pursuant to 21 C.F.R. § 3.2(e) or its foreign equivalent.

1.97 "**[***]**" has the meaning set forth in Section 5.2 (Arrowhead Supply Obligation).

1.98 "**Commercialization**" means any and all activities directed to the marketing, promotion, distribution, offering for sale, sale, having sold, importing, having imported, exporting, having exported, or other commercialization of a pharmaceutical or biologic product, but excluding activities directed to Manufacturing, Development, or Medical Affairs. "**Commercialize**," "**Commercializing**," and "**Commercialized**" will be construed accordingly.

1.99 "**Commercially Reasonable Efforts**" means (a) with respect to the efforts and resources to be expended, or considerations to be undertaken, by Sarepta with respect to any objective or activity related to the Development, Regulatory Approval, Manufacture, Medical Affairs or Commercialization of a Licensed Compound or a Licensed Product, the efforts, resources and considerations to accomplish such objective or activity as Sarepta would normally use to accomplish a similar objective or activity under similar circumstances, it being understood and agreed that such efforts and resources will be consistent with those efforts and resources commonly used by Sarepta under similar circumstances for similar compounds or products owned by it or to which it has similar rights, which compound or product, as applicable, is at a similar stage in its development or product life and of similar market potential, taking into account all relevant factors, including: (i) issues of efficacy, safety, and expected and actual approved labeling, (ii) the expected and actual competitiveness of alternative products sold by Third Parties in the marketplace, (iii) the expected and actual product profile of the Licensed Product, (iv) the expected and actual patent coverage and other proprietary position of the Licensed Product, (v) the likelihood of receiving Regulatory Approval [***] given the regulatory structure involved, including regulatory or data exclusivity, and (vi) the expected and actual profitability [***] of the Licensed Product, and (b) with respect to the efforts and resources to be expended by Arrowhead, with respect to any objective or activity under this Agreement, the efforts and resources to accomplish such objective or activity Arrowhead would normally use to accomplish for its own similar objective under similar

circumstances. Commercially Reasonable Efforts will be determined on a country-by-country and indication-by-indication basis for each Licensed Product, as applicable, and it is anticipated that the level of effort and resources that constitute "**Commercially Reasonable Efforts**" with respect to a particular country or indication may change over time, reflecting changes in the status of each Licensed Product, as applicable, and the country(ies) involved.

1.100 "**Competitive Infringement**" means (a) the making, using, selling, offering for sale, importing, or exporting by a Third Party of a pharmaceutical or biologic product in a country that actually or potentially infringes a Valid Claim of an Arrowhead Patent Right or a Sarepta Arising Patent Right in such country or (b) the filing of an ANDA under Section 505(j) of the FD&C Act or an application under Section 505(b)(2) of the FD&C Act naming a Licensed Product as a reference listed drug and including a certification under Section 505(j)(2)(A)(vii)(IV) or 505(b)(2)(A)(IV), respectively.

1.101 "**Confidential Information**" means (a) the terms of this Agreement and (b) with respect to a Party, subject to Section 9.3 (Exemptions), all Know-How or other information, including proprietary information and materials (whether or not patentable) embodying such Party's technology, products, business information, or objectives, that is communicated by or on behalf of such Party (the "**Disclosing Party**" with respect to such information) to the other Party (the "**Receiving Party**" with respect to such information) or its permitted recipients, including information disclosed by such Party prior to the Effective Date pursuant to the Confidentiality Agreement.

1.102 "**Confidentiality Agreement**" means that certain Mutual Confidential Disclosure Agreement dated [***] by and between Arrowhead and Sarepta, as amended [***].

1.103 "**Control**" or "**Controlled**" means the possession by a Party or its Affiliates (whether by ownership, license, sublicense or otherwise, other than pursuant to this Agreement) of, (a) with respect to any tangible Know-How or materials, the legal authority or right to physical possession of such tangible Know-How or materials, with the right to provide such tangible Know-How or materials to the other Party on the terms set forth herein, (b) with respect to Patent Rights, Regulatory Approvals, Regulatory Submissions, intangible Know-How, or other intellectual property, the legal authority or right to grant a license, sublicense, access, or right to use (as applicable) to the other Party under such Patent Rights, Regulatory Approvals, Regulatory Submissions, intangible Know-How, or other intellectual property on the terms set forth herein, or (c) with respect to a product or component thereof, the legal authority or right to grant a license, sublicense, access, or right to use (as applicable) to the other Party under Patent Rights that Cover, or proprietary Know-How that is incorporated in or embodies, such product or component on the terms set forth herein, in each case ((a), (b), and (c)), without (i) breaching or otherwise violating the terms of any arrangement or agreement with a Third Party in existence as of the time such Party or its Affiliates would first be required hereunder to grant the other Party such access, right to use, license, or sublicense, or (ii) incurring any additional payment obligations to a Third Party that are not subject to an allocation agreed between the Parties pursuant to this Agreement, including in accordance with Section 2.9 (Third Party In-License Payments) or otherwise in writing. Notwithstanding any provision in this Agreement to the contrary, following the closing of a Change of Control of Arrowhead, the Parties agree that Arrowhead will be deemed not to Control any materials, tangible Know-How, Patent Rights, Regulatory Submissions, Regulatory Approvals, intangible Know-How, or other intellectual property that are owned or in-licensed by an Acquirer or any of its Affiliates immediately prior to the closing of such Change of Control, except to the extent such materials, tangible Know-How, Patent Rights, Regulatory Submissions, Regulatory

Approvals, intangible Know-How, or other intellectual property owned or in-licensed by the Acquirer or such Affiliate (A) were included in the licenses or other rights granted to Sarepta pursuant to this Agreement immediately prior to the closing of such Change of Control or (B) are used in the performance of any of Arrowhead's or its Affiliates' obligations, or exercise of its or their rights, under this Agreement following the closing of such Change of Control.

1.104 "**Cover,**" "**Covering**," or "**Covered**" means, with respect to a particular subject matter at issue and a relevant Patent Right or individual claim in such Patent Right, as applicable, that the manufacture, use, sale, offer for sale, or importation of such subject matter would fall within the scope of one or more claims in such Patent Right or the individual claim of such Patent Right.

1.105 "**CRO**" means a contract research organization.

1.106 "**CTA**" has the meaning set forth in Section 1.173 ("IND").

1.107 "**CTA Ready Package Acceptance Date**" means (a) with respect to each Program within the Category 2 Programs, the acceptance date of the Category 2 CTA Ready Package for such Program pursuant to Section 3.1.2(c) (Sarepta Category 2 CTA Ready Package Acceptance), and (b) with respect to each Program within the Category 3 Programs, the acceptance date of the Category 3 CTA Ready Package for such Program pursuant to Section 3.1.3(e) (Sarepta Category 3 CTA Ready Package Acceptance).

1.108 "**CTA Transfer Date**" has the meaning set forth in Section 4.2.1(a) (Clinical Trial Regulatory Submissions).

1.109 "**Cure Period**" has the meaning set forth in Section 13.5.1 (Material Breach and Cure Period).

1.110 "**Debarred**" means, with respect to an individual or entity, that such individual or entity has been debarred or suspended under 21 U.S.C. §335(a) or (b), the subject of a conviction described in Section 306 of the FD&C Act, excluded from a federal or governmental health care program, debarred from federal contracting, convicted of or pled *nolo contendere* to any felony, or to any federal or state legal violation (including misdemeanors) relating to prescription drug products or fraud, the subject of OFAC sanctions or on the OFAC list of specially designated nationals, or the subject of any similar sanction of any Governmental Authority in the Territory.

1.111 "**Delivery Ligand**" means a ligand (including any linkers, whether incorporated into the ligand or a separate component) that is (a) conjugated to an RNAi Molecule to help facilitate delivery *in vivo* to specific tissues or cell types, which may include lipid moieties, antibodies, peptides, and small molecule compounds, (b) a component of, or used in the Manufacture of, Licensed Compounds or Licensed Products, and (c) based on, evolved from, a process improvement to, or is otherwise derived from the Arrowhead Platform, Arrowhead BBB Platform, or Arrowhead Cardiomyocyte Platform.

1.112 "**Development**" means all internal and external research, development, and regulatory activities related to pharmaceutical or biologic products (including Research), including (a) toxicology testing and studies, non-clinical and preclinical testing, studies, and other activities, and Clinical Trials, and (b) preparation, submission, review, and development of data or information for the purpose of submission to a Regulatory Authority to obtain authorization to conduct Clinical Trials and to obtain, support, or maintain Regulatory Approval of a pharmaceutical or biologic product

and interacting with Regulatory Authorities following receipt of Regulatory Approval in the applicable country or region for such pharmaceutical or biologic product regarding the foregoing, but excluding activities directed to Manufacturing, Medical Affairs, or Commercialization. Development will include development and regulatory activities for additional forms, formulations, or indications for a pharmaceutical or biologic product after receipt of Regulatory Approval of such product (including label expansion), including Clinical Trials initiated following receipt of Regulatory Approval or any Clinical Trial to be conducted after receipt of Regulatory Approval that was mandated by the applicable Regulatory Authority as a condition of such Regulatory Approval with respect to an approved formulation or indication (such as post-marketing studies or observational studies, in either case, if required by any Regulatory Authority in any region in the Territory to support or maintain Regulatory Approval for a pharmaceutical or biologic product in such region). "**Develop**," "**Developing**," and "**Developed**" will be construed accordingly.

1.113 "**Development Plans**" has the meaning set forth in Section 3.1.3(d) (Category 3 Development Plans).

1.114 "**Development Report**" has the meaning set forth in Section 3.6.1(b) (Other Activities).

1.115 "**Direct Costs**" means the sum of the following as incurred for the applicable Licensed Compound, Licensed Product, or any other tangible material to be provided by one Party to the other Party hereunder: [***].

1.116 "**Directed To**" means, with respect to a compound or product and a gene target, that the mechanism of such compound or product [***] such target.

1.117 "**Disclosing Party**" has the meaning set forth in Section 1.101 ("Confidential Information").

1.118 "**Disputes**" has the meaning set forth in Section 15.1 (Exclusive Dispute Resolution Mechanism).

1.119 "**[***]**" means [***].

1.120 "**[***]**" means [***].

1.121 "**[***]**" means [***]

1.122 "**[***]**" means, [***].

1.123 "**[***]**" means [***].

1.124 "**[***]**" has the meaning set forth in [***].

1.125 "**[***]**" has the meaning set forth in [***].

1.126 "**DM1 Phase I Clinical Trial**" means the Phase I Clinical Trial titled "A Phase 1/2a Dose-Escalating Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of ARO-DM1 in Subjects With Type 1 Myotonic Dystrophy Who Are ≥18 to ≤ 65 Years" (Clinical Trial ID: NCT06138743).

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1.127 "**DM1 Program**" means the program for the Research, Development, Manufacture, Commercialization, and other Exploitation of Licensed DM1 Compounds and Licensed DM1 Products, but, which for clarity, shall not include the [***].

1.128 "**[***]**" means [***].

1.129 "**[***]**" has the meaning set forth in [***].

1.130 "**DMPK**" means dystrophia myotonica protein kinase, alternatively referred to as DM1 protein kinase.

1.131 "**Dollars**" or "**$**" means the legal tender of the United States of America.

1.132 "**Drug Product**" has the meaning set forth in Section 5.2 (Arrowhead Supply Obligation).

1.133 "**Drug Substance**" has the meaning set forth in Section 5.2 (Arrowhead Supply Obligation).

1.134 "**DSC**" has the meaning set forth in Section 8.3.1(c) (Amendment to Existing Clinical Trial Protocol).

1.135 "**DUX4**" means double homeobox 4.

1.136 "**DUX4 Backup Compound**" means any RNAi Molecule Directed To DUX4 that is Controlled by Arrowhead or any of its Affiliates as of the Execution Date or, subject to Section 2.10 (Exclusivity), during the Term, excluding ARO-DUX4.

1.137 "**DUX4 Phase I Clinical Trial**" means the Phase I Clinical Trial titled "A Phase1/2a Dose-Escalating Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of ARO-DUX4 in Adult Patients With Facioscapulohumeral Muscular Dystrophy Type 1" (Clinical Trial ID: NCT06131983).

1.138 "**DUX4 Program**" means the program for the Research, Development, Manufacture, and Commercialization of Licensed DUX4 Compounds and Licensed DUX4 Products.

1.139 "**Effective Date**" has the meaning set forth in Section 14.1 (Effective Date).

1.140 "**EMA**" means the European Medicines Agency or any successor entity.

1.141 "**Exclusive SM Targets**" has the meaning set forth in Section 1.316 ("Skeletal Muscle Target").

1.142 "**Execution Date**" has the meaning set forth in the preamble.

1.143 "**Executive Officer**" means (a) the Chief Executive Officer of Arrowhead (or an executive officer of Arrowhead designated by the Chief Executive Officer of Arrowhead who has the power and authority to resolve a given Dispute or matter) and (b) the Chief Executive Officer of Sarepta (or an executive officer of Sarepta designated by the Chief Executive Officer who has the power and authority to resolve a given Dispute or matter).

1.144 "**Existing Clinical Trials Protocol**" means, with respect to each of the DUX4 Phase I Clinical Trial, the DM1 Phase I Clinical Trial, the ATXN2 Phase I Clinical Trial, and the MMP7 Phase I

Clinical Trial, the study protocol existing as of the Execution Date, attached hereto as **Schedule 1.144** (Existing Clinical Trials Protocols).

1.145 "**Existing Lead Compounds**" means ARO-ATXN2, ARO-DM1, ARO-DUX4, ARO-MMP7, and ARO-HTT, in each case, individually or collectively as the context requires.

1.146 "**Exploitation**" means to Develop, Manufacture, Commercialize, or otherwise exploit. When used as a verb, to "**Exploit**" means to engage in any of the foregoing activities.

1.147 "**FD&C Act**" means the United States Federal Food, Drug, and Cosmetic Act, 21 U.S.C. § 301 *et seq.*, as amended together with any rules, regulations, and requirements promulgated thereunder.

1.148 "**FDA**" means the United States Food and Drug Administration or any successor agency thereto.

1.149 "**Field**" means human therapeutic uses for the treatment, prevention, or prophylaxis of any disease, disorder, or condition.

1.150 "**First Commercial Sale**" means, on a country-by-country and Licensed Product-by-Licensed Product basis, the first sale under this Agreement by Sarepta or any of its Affiliates or Sublicensees to an end user or prescriber for use, consumption, or resale of such Licensed Product in such country following receipt of Marketing Approval for such Licensed Product in such country. "**First Commercial Sale**" will not include: (a) any distribution or other sale solely for treatment investigational new drug sales, named patient sales, expanded access program, compassionate or emergency use sales, or pre-license sales made for promotional, charitable, or other compassionate purposes, (b) other similar non-commercial uses or sales at or below cost, (c) samples of Licensed Product in reasonable quantities, or (d) sale of a Licensed Product by Sarepta to an Affiliate or a Sublicensee of Sarepta, unless such Affiliate or such Sublicensee is the end user of such Licensed Product, and *provided* that any subsequent sale of such Licensed Product by such Affiliate or such Sublicensee to an end user or prescriber for use, consumption, or resale of such Licensed Product in a country following receipt of Marketing Approval for such Licensed Product in such country will constitute a First Commercial Sale in the applicable country.

1.151 "**Force Majeure**" means any event beyond the reasonable control of the affected Party, including embargoes; war or acts of war, including terrorism, insurrections, riots, or civil unrest; strikes, lockouts, or other labor disturbances (other than strikes, lockouts, or labor disturbances involving such Party's own employees); epidemics, pandemics, the spread of infectious diseases, and quarantines; fire, floods, earthquakes, or other acts of nature; or acts, omissions, or delays in acting by any Governmental Authority.

1.152 "**FTE**" means a qualified full-time person, or more than one person working the equivalent of a full-time person, where "full time" is based upon a total of 1,800 working hours per Calendar Year of scientific or technical work carried out by one or more duly qualified employees of Arrowhead or its Affiliates. Overtime, and work on weekends, holidays, and the like will not be counted with any multiplier (*e.g.* time-and-a-half or double time) toward the number of hours that are used to calculate the FTE contribution. Each employee utilized by Arrowhead or any of its Affiliates in connection with Arrowhead's or such Affiliate's performance under this Agreement may be less than or greater than one FTE based on the hours actually worked by such employee and will be treated as an FTE on a pro rata basis based upon the actual number of such hours worked divided by 1,800.

1.153 "**FTE Costs**" means, for any period, the FTE Rate multiplied by the number of FTEs in such period. FTEs will be pro-rated on a daily basis if necessary.

1.154 "**FTE Rate**" means, for the period commencing on the Effective Date until such time as the Parties agree otherwise, $[***] per year, subject to annual increases beginning on January 1, 2026 to reflect percentage increase in [***], calculated by [***]; *provided*, *however*, that Arrowhead will apply an FTE Rate of $[***] per year solely in the case of its calculation of the FTE Costs included in [***].

1.155 "**Fully Burdened Cost**" means, with respect to a Party and Licensed Compound, Licensed Product, or any other tangible material to be provided by one Party to the other Party hereunder, [***]. All costs and expenses included in this definition will be calculated in accordance with GAAP by such Party on a consistent basis.

1.156 "**[***]**" means [***].

1.157 "**GAAP**" means United States generally accepted accounting principles, which principles are currently used at the relevant time and consistently applied by the applicable Party.

1.158 "**Generic Entry Date**" has the meaning set forth in Section 8.5.2 (Reduction for Generic Competition).

1.159 "**Generic Product**" means, with respect to a Licensed Product in a particular country of the Territory, any product that is approved, or is sought to be approved, in reliance, in whole or in part, on the prior Regulatory Approval (or on safety or efficacy data submitted in support of the prior Regulatory Approval) of such Licensed Product in such country as determined by the applicable Regulatory Authority of such country, including any product authorized for sale (a) in the U.S. pursuant to Section 505(j) of the FD&C Act (21 U.S.C. 355(j)) or Section 505(b)(2) of the FD&C Act (21 U.S.C. 355(b)(2)), as amended from time to time, (b) in countries of the European Economic Area pursuant to Article 10 (but excluding Art. 10(3)), Article 10a, or Article 10b of Parliament and Council Directive 2001/83/EC as amended from time to time (including an application under Article 6.1 of Parliament and Council Regulation (EC) No. 726/2004 that relies for its content on any such provision), or (c) in any other country or other jurisdiction pursuant to all equivalents of such provisions, including any amendments and successor statutes with respect to any of the foregoing.

1.160 "**Good Clinical Practices**" or "**GCP**" means the then-current good clinical practice standards, practices, and procedures promulgated or endorsed by the applicable Regulatory Authority as set forth in the guidelines imposed by such Regulatory Authority, as may be updated from time-to-time, including those as set forth in FDA regulations in 21 C.F.R. Parts 11, 50, 54, 56, 312, 314, and 320 and all related FDA rules, regulations, orders, and guidances, and by the International Conference on Harmonization E6: Good Clinical Practices Consolidated Guideline.

1.161 "**Good Laboratory Practices,**" or "**GLP**," means the then-current and phase appropriate standards, practices and procedures promulgated or endorsed by the FDA as set forth in 21 C.F.R. Part 58 (or any successor statute or regulation) and FDA guidance, including related regulatory requirements imposed by the FDA and comparable applicable regulatory standards, practices and procedures promulgated by the EMA, PMDA, or other Regulatory Authority applicable to the

Territory, as they may be updated from time to time, including applicable guidelines promulgated under the ICH.

1.162 "**Good Manufacturing Practices,**" or "**GMP**" means the then-current good manufacturing practices required by the FDA, as set forth in the FD&C Act, 21 C.F.R. Parts 210 and 211, and FDA guidance issued thereunder, for the Manufacture and testing of pharmaceutical materials, and comparable applicable Law related to the manufacture and testing of pharmaceutical materials in jurisdictions outside the United States. "**Good Manufacturing Practices**," or "**GMP**" also means the quality guidelines promulgated by the ICH, including the ICH Q7A, titled "Q7A Good Manufacturing Practice Guidance for Active Pharmaceutical Ingredients" and the policies promulgated thereunder, in each case, as they may be updated from time to time.

1.163 "**Governmental Authority**" means any court, tribunal, arbitrator, agency, commission, department, ministry, official, authority, or other instrumentality of any nation, state, county, city, or other political subdivision thereof or of any multinational governmental body.

1.164 "**[***]**" means [***].

1.165 "**Greater China**" means the People's Republic of China, the Special Administrative Region of Hong Kong, the Special Administrative Region of Macau, and Taiwan.

1.166 "**H-W Suit Notice**" has the meaning set forth in Section 12.3.4 (Hatch-Waxman).

1.167 "**Hatch-Waxman Act**" means rights conferred in the U.S. under the Drug Price Competition and Patent Term Restoration Act, 21 U.S.C. §355, as amended (or any successor statute or regulation).

1.168 "**HSR Act**" means the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and the rules promulgated thereunder.

1.169 "**HTT**" means Huntingtin.

1.170 "**HTT Backup Compound**" means any RNAi Molecule Directed To HTT that is Controlled by Arrowhead or any of its Affiliates as of the Execution Date or, subject to Section 2.10 (Exclusivity), during the Term, excluding ARO-HTT.

1.171 "**HTT Program**" means the program for the Research, Development, Manufacture, Commercialization, and other Exploitation of Licensed HTT Compounds and Licensed HTT Products.

1.172 "**ICH**" means International Conference on Harmonization.

1.173 "**IND**" means (a) an Investigational New Drug application pursuant to the FD&C Act, as amended, and applicable regulations promulgated thereunder by the FDA, (b) a Clinical Trial authorization application for a product filed with a Regulatory Authority in any other regulatory jurisdiction outside the U.S., the filing of which is necessary to commence or conduct clinical testing of a pharmaceutical or biologic product in humans in such jurisdiction ("**CTA**"), or (c) documentation issued by a Regulatory Authority that permits the conduct of clinical testing of a pharmaceutical or biologic product in humans in such jurisdiction.

1.174 "**Indemnified Party**" has the meaning set forth in Section 11.3.1 (Notice).

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1.175 "**Indemnifying Party**" has the meaning set forth in Section 11.3.1 (Notice).

1.176 "**Indirect Costs**" means the sum of the following as incurred for the applicable Licensed Compound, Licensed Product, or any other tangible material to be provided by one Party to the other Party hereunder: [***].

1.177 "**[***]**" has the meaning set forth in [***].

1.178 "**JDC Communication Plan**" has the meaning set forth in Section 7.3.3 (Meetings).

1.179 "**JMC Communication Plan**" has the meaning set forth in Section 7.4.3 (Meetings).

1.180 "**Joint Arising Know-How**" has the meaning set forth in Section 12.1.2(c) (Joint).

1.181 "**Joint Arising Patent Rights**" has the meaning set forth in Section 12.1.2(c) (Joint).

1.182 "**Joint Arising Technology**" has the meaning set forth in Section 12.1.2(c) (Joint).

1.183 "**Joint Development Committee**" or "**JDC**" has the meaning set forth in Section 7.3.1 (Formation; Composition; Dissolution).

1.184 "**Joint Manufacturing Committee**" or **"JMC**" has the meaning set forth in Section 7.4.1 (Formation; Composition; Dissolution).

1.185 "**Joint Steering Committee**" or **"JSC**" has the meaning set forth in Section 7.2.1 (Formation; Composition; Dissolution).

1.186 "**Know-How**" means any (a) proprietary scientific or business information or materials, including records, improvements, modifications, techniques, assays, designs, protocols, formulas, data (including physical data, chemical data, toxicology data, animal data, raw data, clinical data, and analytical and quality control data), dosage regimens, control assays, product specifications, marketing, pricing and distribution costs, inventions, algorithms, technology, forecasts, profiles, strategies, plans, results in any form whatsoever, know-how, and trade secrets (in each case, whether or not patentable, copyrightable, or otherwise protectable), and (b) any information embodied in chemical or biological materials or physical embodiments of any of the foregoing.

1.187 "**Laws**" means applicable laws, statutes, rules, regulations, and other pronouncements having the effect of law of any Governmental Authority that may be in effect from time to time, including disclosure obligations required by any stock exchange or securities commission having authority over a Party and any applicable rules, regulations, guidances, or other requirements of any Regulatory Authority that may be in effect from time to time.

1.188 "**Licensed ATXN1 Compound**" means any and all of the following (a) ARO-ATXN1, (b) the ATXN1 Backup Compounds, and (c) any modification, improvement, or other derivative to or of ARO-ATXN1 or any ATXN1 Backup Compound that is Directed to ATXN1.

1.189 "**Licensed ATXN1 Product**" means any pharmaceutical or biologic product that is comprised of or contains a Licensed ATXN1 Compound, alone or in combination with one or more Other Components, in any and all forms, presentations, delivery systems, dosages, and formulations and any improved or modified versions thereof.

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1.190 "**Licensed ATXN1 Product-Specific Patent Rights**" means all Arrowhead Patent Rights having claims Covering solely (a) the composition of matter comprising the nucleotide sequence of one or more Licensed ATXN1 Compounds or Licensed ATXN1 Products, (b) the method of use (including method of treatment by use) of one or more Licensed ATXN1 Compounds or Licensed ATXN1 Products, (c) the formulation comprising, and biomarkers or companion diagnostics specifically relating to, one or more Licensed ATXN1 Compounds or Licensed ATXN1 Products, or (d) the method of manufacture specific to the Manufacture of Licensed ATXN1 Compounds or Licensed ATXN1 Products.

1.191 "**Licensed ATXN2 Compound**" means any and all of the following (a) ARO-ATXN2, (b) the ATXN2 Backup Compounds, and (c) any modification, improvement, or other derivative to or of ARO-ATXN2 or any ATXN2 Backup Compound that is Directed to ATXN2.

1.192 "**Licensed ATXN2 Product**" means any and all of the following (a) the product containing ARO-ATXN2, which is the subject of the ATXN2 Phase I Clinical Trial as of the Execution Date, and (b) any pharmaceutical or biologic product that is comprised of or contains a Licensed ATXN2 Compound, alone or in combination with one or more Other Components, in any and all forms, presentations, delivery systems, dosages, and formulations and any improved or modified versions thereof.

1.193 "**Licensed ATXN2 Product-Specific Patent Rights**" means all Arrowhead Patent Rights having claims Covering solely (a) the composition of matter comprising the nucleotide sequence of one or more Licensed ATXN2 Compounds or Licensed ATXN2 Products, (b) the method of use (including method of treatment by use) of one or more Licensed ATXN2 Compounds or Licensed ATXN2 Products, (c) the formulation comprising, and biomarkers or companion diagnostics specifically relating to, one or more Licensed ATXN2 Compounds or Licensed ATXN2 Products, or (d) the method of manufacture specific to the Manufacture of Licensed ATXN2 Compounds or Licensed ATXN2 Products.

1.194 "**Licensed ATXN3 Compound**" means any and all of the following (a) ARO-ATXN3, (b) the ATXN3 Backup Compounds, and (c) any modification, improvement, or other derivative to or of ARO-ATXN3 or any ATXN3 Backup Compound that is Directed to ATXN3.

1.195 "**Licensed ATXN3 Product**" means any pharmaceutical or biologic product that is comprised of or contains a Licensed ATXN3 Compound, alone or in combination with one or more Other Components, in any and all forms, presentations, delivery systems, dosages, and formulations and any improved or modified versions thereof.

1.196 "**Licensed ATXN3 Product-Specific Patent Rights**" means all Arrowhead Patent Rights having claims Covering solely (a) the composition of matter comprising the nucleotide sequence of one or more Licensed ATXN3 Compounds or Licensed ATXN3 Products, (b) the method of use (including method of treatment by use) of one or more Licensed ATXN3 Compounds or Licensed ATXN3 Products, (c) the formulation comprising, and biomarkers or companion diagnostics specifically relating to, one or more Licensed ATXN3 Compounds or Licensed ATXN3 Products, or (d) the method of manufacture specific to the Manufacture of Licensed ATXN3 Compounds or Licensed ATXN3 Products.

1.197 "**Licensed C1 Compounds**" means the Licensed ATXN2 Compounds, the Licensed DM1 Compounds, the Licensed DUX4 Compounds, and the Licensed MMP7 Compounds, in each case, individually or collectively as the context requires.

1.198 "**Licensed C1 Products**" means the Licensed ATXN2 Products, the Licensed DM1 Products, the Licensed DUX4 Products, and the Licensed MMP7 Products, in each case, individually or collectively as the context requires.

1.199 "**Licensed C2 Compounds**" means the Licensed ATXN1 Compounds, the Licensed ATXN3 Compounds, and the Licensed HTT Compounds, in each case, individually or collectively as the context requires.

1.200 "**Licensed C2 Products**" means the Licensed ATXN1 Products, the Licensed ATXN3 Products, and the Licensed HTT Products, in each case, individually or collectively as the context requires.

1.201 "**Licensed C3 Compounds**" means, on a Category 3 Program-by-Category 3 Program basis, (a) the lead RNAi Molecule Directed To the Collaboration Target of such Category 3 Program that is the subject of the Category 3 CTA Ready Package accepted by Sarepta pursuant to 3.1.3(e) (Sarepta Category 3 CTA Ready Package Acceptance), (b) any backups of the RNAi Molecule described in clause (a) Controlled by Arrowhead or any of its Affiliates as of the CTA Ready Package Acceptance Date, and (c) any modification, improvement, or other derivative to or of the RNAi Molecules described in clauses (a) or (b).

1.202 "**Licensed C3 Product-Specific Patent Rights**" means all Arrowhead Patent Rights having claims Covering solely (a) the composition of matter comprising the nucleotide sequence of one or more Licensed C3 Compounds or Licensed C3 Products, (b) the method of use (including method of treatment by use) of one or more Licensed C3 Compounds or Licensed C3 Products, (c) the formulation comprising, and biomarkers or companion diagnostics specifically relating to, one or more Licensed C3 Compounds or Licensed C3 Products, or (d) the method of manufacture specific to the Manufacture of Licensed C3 Compounds or Licensed C3 Products.

1.203 "**Licensed C3 Products**" means any pharmaceutical or biologic product that is comprised of or contains a Licensed C3 Compound, alone or in combination with one or more Other Components, in any and all forms, presentations, delivery systems, dosages, and formulations and any improved or modified versions thereof.

1.204 "**Licensed Compounds**" means the Licensed C1 Compounds, the Licensed C2 Compounds, and the Licensed C3 Compounds, in each case, individually or collectively as the context requires.

1.205 "**Licensed DM1 Compound**" means any and all of the following (a) ARO-DM1, (b) the [***], and (c) any modification, improvement, or other derivative to or of ARO-DM1 or any [***] that is Directed to DM1.

1.206 "**Licensed DM1 Product**" means any pharmaceutical or biologic product that is comprised of or contains a Licensed DM1 Compound, alone or in combination with one or more Other Components, in any and all forms, presentations, delivery systems, dosages, and formulations and any improved or modified versions thereof.

1.207 "**Licensed DM1 Product-Specific Patent Rights**" means all Arrowhead Patent Rights having claims Covering solely (a) the composition of matter comprising the nucleotide sequence of one or

more Licensed DM1 Compounds or Licensed DM1 Products, (b) the method of use (including method of treatment by use) of one or more Licensed DM1 Compounds or Licensed DM1 Products, (c) the formulation comprising, and biomarkers or companion diagnostics specifically relating to, one or more Licensed DM1 Compounds or Licensed DM1 Products, or (d) the method of manufacture specific to the Manufacture of Licensed DM1 Compounds or Licensed DM1 Products.

1.208 "**Licensed DUX4 Compound**" means any and all of the following (a) ARO-DUX4, (b) the DUX4 Backup Compounds, and (c) any modification, improvement, or other derivative to or of ARO-DUX4 or any DUX4 Backup Compound that is Directed To DUX4.

1.209 "**Licensed DUX4 Product**" means any pharmaceutical or biologic product that is comprised of or contains a Licensed DUX4 Compound, alone or in combination with one or more Other Components, in any and all forms, presentations, delivery systems, dosages, and formulations and any improved or modified versions thereof.

1.210 "**Licensed DUX4 Product-Specific Patent Rights**" means all Arrowhead Patent Rights having claims Covering solely (a) the composition of matter comprising the nucleotide sequence of one or more Licensed DUX4 Compounds or Licensed DUX4 Products, (b) the method of use (including method of treatment by use) of one or more Licensed DUX4 Compounds or Licensed DUX4 Products, (c) the formulation comprising, and biomarkers or companion diagnostics specifically relating to, one or more Licensed DUX4 Compounds or Licensed DUX4 Products, or (d) the method of manufacture specific to the Manufacture of Licensed DUX4 Compounds or Licensed DUX4 Products.

1.211 "**Licensed HTT Compound**" means any and all of the following (a) ARO-HTT, (b) the HTT Backup Compounds, and (c) any modification, improvement, or other derivative to or of ARO-HTT or any HTT Backup Compound that is Directed to HTT.

1.212 "**Licensed HTT Product**" means any pharmaceutical or biologic product that is comprised of or contains a Licensed HTT Compound, alone or in combination with one or more Other Components, in any and all forms, presentations, delivery systems, dosages, and formulations and any improved or modified versions thereof.

1.213 "**Licensed HTT Product-Specific Patent Rights**" means all Arrowhead Patent Rights having claims Covering solely (a) the composition of matter comprising the nucleotide sequence of one or more Licensed HTT Compounds or Licensed HTT Products, (b) the method of use (including method of treatment by use) of one or more Licensed HTT Compounds or Licensed HTT Products, (c) the formulation comprising, and biomarkers or companion diagnostics specifically relating to, one or more Licensed HTT Compounds or Licensed HTT Products, or (d) the method of manufacture specific to the Manufacture of Licensed HTT Compounds or Licensed HTT Products.

1.214 "**Licensed MMP7 Compound**" means any and all of the following (a) ARO-MMP7, (b) the MMP7 Backup Compounds, and (c) any modification, improvement, or other derivative to, or of, ARO-MMP7 or any MMP7 Backup Compound that is Directed to MMP7.

1.215 "**Licensed MMP7 Product**" means any pharmaceutical or biologic product that is comprised of or contains a Licensed MMP7 Compound, alone or in combination with one or more Other Components, in any and all forms, presentations, delivery systems, dosages, and formulations and any improved or modified versions thereof.

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1.216 "**Licensed MMP7 Product-Specific Patent Rights**" means all Arrowhead Patent Rights having claims Covering solely (a) the composition of matter comprising the nucleotide sequence of one or more Licensed MMP7 Compounds or Licensed MMP7 Products, (b) the method of use (including method of treatment by use) of one or more Licensed MMP7 Compounds or Licensed MMP7 Products, (c) the formulation comprising, and biomarkers or companion diagnostics specifically relating to, one or more Licensed MMP7 Compounds or Licensed MMP7 Products, or (d) the method of manufacture specific to the Manufacture of Licensed MMP7 Compounds or Licensed MMP7 Products.

1.217 "**Licensed Product-Specific Patent Rights**" means, collectively, the Licensed DM1 Product-Specific Patent Rights, the Licensed DUX4 Product-Specific Patent Rights, the Licensed ATXN2 Product-Specific Patent Rights, the Licensed MMP7 Product-Specific Patent Rights, the Licensed ATXN1 Product-Specific Patent Rights, the Licensed ATXN3 Product-Specific Patent Rights, the Licensed HTT Product-Specific Patent Rights, and the Licensed C3 Product-Specific Patent Rights. The Licensed Product-Specific Patent Rights existing as of the Execution Date are set forth on **Schedule 1.217** (Licensed Product-Specific Patent Rights).

1.218 "**Licensed Products**" means the Licensed C1 Products, the Licensed C2 Products, and the Licensed C3 Products, in each case, individually or collectively as the context requires.

1.219 "**Losses**" has the meaning set forth in Section 11.1 (Indemnification by Arrowhead).

1.220 "**MAA**" means any new drug application or other marketing authorization application, in each case, filed with the applicable Regulatory Authority in a country or other regulatory jurisdiction (and all supplements and amendments thereto), which application is required to commercially market or sell a pharmaceutical or biologic product in such country or jurisdiction, including all New Drug Applications submitted to the FDA in the United States pursuant to the FD&C Act (21 U.S.C. § 355(b)(1)) and the regulations promulgated thereunder with respect to a pharmaceutical product or any analogous application or submission with any Regulatory Authority in any other country or regulatory jurisdiction.

1.221 "**Major European Markets**" means the United Kingdom, France, Germany, Italy, and Spain.

1.222 "**Major Region**" means (a) the United States, (b) all of the Major European Markets, (c) Japan, or (d) Greater China.

1.223 "**Manufacture**" means activities directed to manufacturing, processing, CMC Activities, packaging, labeling, filling, finishing, assembly, testing, release, shipping, or storage of any pharmaceutical or biologic product (or any components or process steps involving any product or any companion diagnostic), placebo, or comparator agent, as the case may be, but excluding activities directed to Development, Commercialization, or Medical Affairs. "**Manufacturing**" will be construed accordingly.

1.224 "**Manufacturing Technology Transfer Plan**" has the meaning set forth in Section 5.4 (Manufacturing Technology Transfer).

1.225 "**[***]**" means [***].

1.226 "**Marketing Approval**" means, with respect to a country or extra-national territory, any and all approvals (including Regulatory Approval and, if applicable, Pricing and Reimbursement Approval

(including as further described in Section 1.270 ("Pricing and Reimbursement Approval")), licenses, registrations, or authorizations of any Governmental Authority that are required in order to Commercialize a Licensed Product in such country or some or all of such extra-national territory.

1.227 "**Material Trial Change**" means, in each case, with respect to an Ongoing Clinical Trial, [***].

1.228 "**Materials**" means all tangible compositions of matter, devices, articles of manufacture, assays, biological, chemical or physical materials, and other similar materials.

1.229 "**[***]**" means [***].

1.230 "**Medical Affairs**" means activities conducted by a Party's medical affairs departments (or, if a Party does not have a medical affairs department, the equivalent function thereof), including communications with key opinion leaders, medical education, symposia, advisory boards (to the extent related to medical affairs or clinical guidance), activities performed in connection with patient registries, and other medical programs and communications, including educational grants, research grants (including conducting investigator-initiated studies), and charitable donations to the extent related to medical affairs and not to other activities that do not involve the promotion, marketing, sale, or other Commercialization of the Licensed Products and are not conducted by a Party's medical affairs (or equivalent) departments.

1.231 "**[***]**" means [***].

1.232 "**Milestone Events**" means [***], Regulatory Milestone Events, and Sales Milestone Events.

1.233 "**Milestone Payments**" means the [***], Regulatory Milestone Payments, and Sales Milestone Payments.

1.234 "**[***]**" means [***].

1.235 "**MMP7**" means matrix metallopeptidase 7.

1.236 "**MMP7 Backup Compound**" means any RNAi Molecule Directed To MMP7 that is Controlled by Arrowhead or any of its Affiliates as of the Execution Date or, subject to Section 2.10 (Exclusivity), during the Term, excluding ARO-MMP7.

1.237 "**MMP7 Phase I Clinical Trial**" means the Phase I Clinical Trial titled "A Phase 1/2a Study Evaluating the Effects of ARO-MMP7 Inhalation Solution in Healthy Subjects and Patients With Idiopathic Pulmonary Fibrosis" (Clinical Trial ID: NCT05537025).

1.238 "**MMP7 Program**" means the program for the Research, Development, Manufacture, Commercialization, and other Exploitation of Licensed MMP7 Compounds and Licensed MMP7 Products.

1.239 "**Net Sales**" means the gross amounts invoiced by Sarepta or any of its Affiliates or Sublicensees (other than Third Party Distributors) (each, a "**Selling Party**") under this Agreement to Third Parties (including to Third Party Distributors), for the sale, supply, or other disposition of a Licensed Product, less the following deductions actually taken, paid, accrued, allowed, included, or allocated with respect to such sale, supply or other disposition of such Licensed Product, and as determined in accordance with GAAP:

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[***]

Notwithstanding the foregoing, amounts received or invoiced by Sarepta, or its Affiliates, or their respective Sublicensees for the sale of such Licensed Product among Sarepta or its Affiliates, or their respective Sublicensees, for resale will not be included in the computation of Net Sales hereunder (unless such Affiliate or such Sublicensee is the end user of such Licensed Product), and Net Sales will be calculated on the value charged or invoiced on the first arm's-length sale thereafter to a Third Party. In any event, any amounts received or invoiced by Sarepta or its Affiliates, or their respective Sublicensees, will be accounted for only once. For purposes of determining Net Sales, a Licensed Product will be deemed to be sold when recorded as a sale by Sarepta or its Affiliates, or their respective Sublicensees, in accordance with GAAP. For clarity, a particular deduction may only be accounted for once in the calculation of Net Sales. Net Sales will exclude (a) any distribution or other sale solely for treatment investigational new drug sales, named patient sales, expanded access program, compassionate or emergency use sales, or pre-license sales made for promotional, charitable or other compassionate purposes, (b) other similar non-commercial uses or sales at or below cost, or (c) samples of Licensed Product in reasonable quantities.

To the extent that Sarepta or any of its Affiliates or any of their respective Sublicensees receives consideration other than or in addition to cash upon the sale or disposition of a Licensed Product, Net Sales will be calculated based on the average price charged for such Licensed Product, as applicable, during the preceding royalty period, or, in the absence of such sales, based on Sarepta's or such Affiliate's or such Sublicensee's reasonable determination of the fair market value of such Licensed Product. The permitted deductions of clauses (a) through (g) above will be fairly allocated to such Licensed Product and will not be inappropriately allocated as between such Licensed Product and other products or services of Sarepta or such Affiliate or such Sublicensee.

In the case of any Combination Product sold in a given country in the Territory, Net Sales for the purpose of determining Royalties and Sales Milestone Events of such Combination Product in such country will be calculated by multiplying actual Net Sales of such Combination Product in such country by the fraction A/(A+B), where A is the invoice price of the Licensed Product that includes the same Licensed Compound as the Combination Product, if sold separately as a stand-alone Licensed Product in such country, and B is the total invoice price of the Other Components in the Combination Product, if sold separately in such country.

If, on a country-by-country basis, a Licensed Product that includes the same Licensed Compound as the Combination Product is sold separately as a stand-alone Licensed Product in a country, but the Other Components in such Combination Product are not sold separately in such country, then Net Sales for the purpose of determining Royalties and Sales Milestone Events of such Combination Product for such country will be calculated by multiplying actual Net Sales of such Combination Product in such country by the fraction A/C, where A is the invoice price of the Licensed Product that includes the same Licensed Compound as the Combination Product if sold separately as a stand-alone Licensed Product in such country, and C is the invoice price of such Combination Product in such country.

If, on a country-by-country basis, a Licensed Product that includes the same Licensed Compound as the Combination Product is not sold separately as a stand-alone Licensed Product in such country, but the Other Components included in such Combination Product are sold separately in such country, then Net Sales for the purpose of determining Royalties and Sales Milestone

Events of such Combination Product for such country will be calculated by multiplying actual Net Sales of such Combination Product in such country by the fraction C-B/C, where B is the invoice price of the Other Components included in such Combination Product if sold separately in such country, and C is the invoice price of such Combination Product in such country.

If neither the Licensed Product that includes the same Licensed Compound as the Combination Product nor the Other Components included in such Combination Product are sold separately in a given country, then Net Sales for the purpose of determining Royalties and Sales Milestone Events in such country will be calculated based on the Parties' good faith agreement as to the estimate of the fair market value of the Licensed Compound and each of the Other Components included in such Combination Product when sold in such country. If the Parties do not so agree, either Party will have the right to refer such matter to be determined by the expedited arbitration procedure set forth in Section 15.3 (Expedited Arbitration).

1.240 "**Non-Breaching Party**" has the meaning set forth in Section 13.5.1 (Material Breach and Cure Period).

1.241 "**Non-Exclusive SM Targets**" has the meaning set forth in Section 1.316 ("Skeletal Muscle Target").

1.242 "**[***] Agreement**" means [***].

1.243 "**OFAC**" means the Office of Foreign Assets Control of the United States Department of the Treasury or any successor agency thereto.

1.244 "**Ongoing C1 Development Activities**" means all Development activities in support of the Ongoing Development Trials as set forth in the Category 1 Development Plans, other than those activities designated under the Category 1 Development Plans to be performed by Sarepta.

1.245 "**Ongoing C1 Development Activities Cure Period**" has the meaning set forth in Section 3.1.1(b) (Sarepta Category 1 Development Step-In Right).

1.246 "**Ongoing Clinical Trials**" means the DUX4 Phase I Clinical Trial, the DM1 Phase I Clinical Trial [***], the ATXN2 Phase I Clinical Trial, and the MMP7 Phase I Clinical Trial, in each case, individually or collectively as the context requires.

1.247 "**Ongoing Development Trials**" means (a) the Ongoing Clinical Trials and (b) those other non-clinical studies that are ongoing as of the Effective Date, as set forth on **Schedule 1.247** (Ongoing Development Trials).

1.248 "**Other Component**" has the meaning set forth in Section 1.96 ("Combination Product").

1.249 "**Out-of-Pocket Costs**" means, with respect to certain activities for a Licensed Compound or Licensed Product hereunder, specifically identifiable expenses paid or payable by a Party or its Affiliates to Third Parties to conduct such activities, including payments to contract personnel (including contractors, consultants, and Subcontractors).

1.250 "**Party**" or "**Parties**" has the meaning set forth in the preamble.

1.251 "**Patent Challenge**" has the meaning set forth in Section 13.6 (Termination for Patent Challenge).

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1.252 "**Patent Costs**" means the Out-of-Pocket Costs paid to outside legal counsel and other Third Parties incurred in the Prosecution and Maintenance of Patent Rights hereunder or enforcing and defending any such Patent Rights, determining freedom to operate for any Licensed Products (including challenging any Patent Right Controlled by Third Parties).

1.253 "**Patent Offices**" has the meaning set forth in Section 10.2.7 (Validity and Enforceability).

1.254 "**Patent Right**" means any and all (a) patents, (b) patent applications, including all provisional and non-provisional applications, patent cooperation treaty (PCT) applications, substitutions, continuations, continuations-in-part, divisions and renewals, and all patent rights granted thereon or claiming priority thereto, (c) all patents-of-addition, reissues, re-examinations and extensions or restorations by existing or future extension or restoration mechanisms, including supplementary protection certificates, patent term extensions, and equivalents thereof, (d) inventor's certificates, letters patent, (e) any other substantially equivalent form of government issued right substantially similar to any of the foregoing described in subsections (a) through (e) above, anywhere in the world.

1.255 "**Patent Term Extensions**" has the meaning set forth in Section 12.6 (Patent Right Extensions).

1.256 "**Payments**" has the meaning set forth in Section 8.7.5(a) (Withholding Taxes).

1.257 "**[***]**" means [***].

1.258 "**Person**" means any natural person, corporation, unincorporated organization, partnership, association, sole proprietorship, joint stock company, joint venture, limited liability company, trust or government, Governmental Authority, or any other similar entity.

1.259 "**Personnel**" means, with respect to any Person, its officers, directors, employees, workers, contractors, advisors, consultants, agents, or other representatives.

1.260 "**Pharmacovigilance Agreement**" has the meaning set forth in Section 4.4 (Pharmacovigilance Agreement).

1.261 "**Phase I Clinical Trial**" means a Clinical Trial (or any arm thereof) of a pharmaceutical or biologic product with the endpoint of determining initial tolerance, safety, metabolism, pharmacokinetic or pharmacodynamic information in single dose, single ascending dose, multiple dose, or multiple ascending dose regimens, and that satisfies the requirements of U.S. federal regulation 21 C.F.R. §§ 312.21(a) and its successor regulation or equivalents in other jurisdictions.

1.262 "**Phase II Clinical Trial**" means a Clinical Trial (or any arm thereof) of a pharmaceutical or biologic product with the primary objective of characterizing its effectiveness in a specific disease state as well as generating more detailed safety, tolerability, and pharmacokinetics information, and that satisfies the requirements of U.S. federal regulation 21 C.F.R. §§ 312.21(b) and its successor regulation or equivalents in other jurisdictions.

1.263 "**Phase III Clinical Trial**" means a Clinical Trial (or any arm thereof) of a pharmaceutical or biologic product on a sufficient number of patients, which trial a Regulatory Authority permits to be conducted under an open IND and is designed to: (a) establish that the pharmaceutical or biologic product is safe and efficacious for its intended use; (b) define warnings, precautions and adverse reactions that are associated with the pharmaceutical or biologic product in the dosage range to be

prescribed; and (c) support an MAA filed with a Regulatory Authority for the pharmaceutical or biologic product, and that satisfies the requirements of U.S. federal regulation 21 C.F.R. § 312.21(c) and its successor regulation or equivalents in other jurisdictions.

1.264 "**Platform Third Party Agreements**" has the meaning set forth in Section 2.9.2(b)(i) (Platform Third Party Rights).

1.265 "**Platform Third Party Rights**" has the meaning set forth in Section 2.9.2(b)(i) (Platform Third Party Rights).

1.266 "**[***]**" means [***].

1.267 "**[***]**" has the meaning set forth in [***].

1.268 "**Pre-Existing Third Party Agreements**" means those certain agreements between Arrowhead and a Third Party set forth on **Schedule 1.268** (Pre-Existing Third Party Agreements).

1.269 "**Price Applicability Period**" has the meaning set forth in Section 1191(b)(2) of the Social Security Act.

1.270 "**Pricing and Reimbursement Approval**" means the later of (a) the approval, agreement, determination, or governmental decision establishing a price for the applicable Licensed Product that can be legally charged to consumers, if required in a given jurisdiction or country in connection with Commercialization of such Licensed Product in such jurisdiction or country; and (b) the approval, agreement, determination, or governmental decision establishing the level of reimbursement for the applicable Licensed Product that will be reimbursed by Governmental Authorities, if required in a given jurisdiction or country in connection with the Commercialization of such Licensed Product in such jurisdiction or country. For purposes of this definition and its use in Section 1.226 ("Marketing Approval"), [***].

1.271 "**Product-Specific Know-How**" has the meaning set forth in Section 9.1 (Confidential Information).

1.272 "**Program**" means each of the Category 1 Programs, the Category 2 Programs, and the Category 3 Programs.

1.273 "**Program-Specific Third Party Rights**" has the meaning set forth in Section 2.9.2(a) (Program-Specific Third Party Rights).

1.274 "**Prosecution and Maintenance**" or "**Prosecute and Maintain**" means the filing, preparation, prosecution (including any interferences, reissue proceedings, reexaminations, oppositions and similar proceedings), post-grant reviews, requests for patent term adjustments, and maintenance of Patent Rights. For the avoidance of doubt, Prosecution and Maintenance excludes any applications or requests for patent term extension. When used as a verb, "**Prosecute and Maintain**" means to engage in Prosecution and Maintenance.

1.275 "**Receiving Party**" has the meaning set forth in Section 1.101 ("Confidential Information").

1.276 "**Redacted Agreement**" has the meaning set forth in Section 9.5 (Confidential Treatment).

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1.277 "**Regulatory Approval**" means, with respect to a particular country or other regulatory jurisdiction, any approval of an MAA, or other approval, product, or establishment license, registration, or authorization of any Regulatory Authority necessary for the Manufacture, Commercialization, or other Exploitation of a pharmaceutical or biologic product in such country or other regulatory jurisdiction, including all supplements and amendments thereto, excluding, in each case, Pricing and Reimbursement Approval.

1.278 "**Regulatory Authority**" means any applicable Governmental Authority with jurisdiction or authority over the Development, Manufacture, Commercialization, or other Exploitation (including Marketing Approval, Regulatory Approval, or Pricing and Reimbursement Approval) of pharmaceutical or biologic products in a particular country or other regulatory jurisdiction, and any corresponding national or regional regulatory authorities.

1.279 "**Regulatory Exclusivity**" means any exclusive marketing rights or data exclusivity rights conferred by any Regulatory Authority with respect to a Licensed Product in a country or jurisdiction in the Territory, other than a Patent Right, that prohibits a Person from relying on or otherwise using safety or efficacy data generated by or on behalf of a Party with respect to such Licensed Product, including new use or indication exclusivity, new formulation, new chemical entity exclusivity, orphan drug exclusivity, or non-Patent related pediatric exclusivity.

1.280 "**Regulatory Lead**" means Sarepta, *except* with respect to any Regulatory Submissions for each Licensed C1 Product prior to the CTA Transfer Date for such product, for which Arrowhead will be the Regulatory Lead; *provided* that [***].

1.281 "**Regulatory Milestone Event**" has the meaning set forth in Section 8.3.2 (Regulatory Milestones).

1.282 "**Regulatory Milestone Payment**" has the meaning set forth in Section 8.3.2 (Regulatory Milestones).

1.283 "**Regulatory Submissions**" means any filing, application, dossier, or submission with any Regulatory Authority in support of the Development, Manufacture, Commercialization, or other Exploitation of a pharmaceutical or biologic product (including to obtain, support, or maintain Regulatory Approval from that Regulatory Authority), including all supplements, amendments, data, and documents with respect thereto, and all correspondence or communication with or from the relevant Regulatory Authority, as well as minutes of any material meetings, telephone conferences, or discussions with the relevant Regulatory Authority. Regulatory Submissions include all INDs, MAAs, and other applications for Regulatory Approval and their equivalents.

1.284 "**Reimbursable Development Costs**" has the meaning set forth in Section 3.4.1 (Arrowhead Development Costs Reimbursement).

1.285 "**[***]**" means [***].

1.286 "**Replacement Target**" has the meaning set forth in Section 3.1.3(b)(iv) (Sarepta Collaboration Target Substitution Right).

1.287 "**Research**" means all internal and external research, identification of composition of matter, screening, and non-human testing, including all non-clinical toxicology testing and studies, non-clinical and preclinical testing, studies, and other activities. When used as a verb, "**to Research**" and "**Researching**" mean to engage or engaging in Research.

1.288 "**Restricted Party**" means any individual or entity on one or more of the Restricted Party Lists.

1.289 "**Restricted Party List**" means the list of sanctioned entities maintained by the United Nations; the Specially Designated Nationals and Blocked Persons List, the Foreign Sanctions Evaders List and the Sectoral Sanctions Identifications List, all administered by OFAC; the U.S. Denied Persons List, the U.S. Entity List, and the U.S. Unverified List, all administered by the U.S. Department of Commerce; and the entities subject to restrictive measures and the consolidated list of Persons, Groups, and Entities Subject to E.U. Financial Sanctions, as implemented by the E.U. Common Foreign & Security Policy.

1.290 "**Reversion License**" has the meaning set forth in Section 13.7.3 (Reversion License).

1.291 "**Reversion Trademarks**" has the meaning set forth in Section 13.7.10 (Sarepta Trademarks).

1.292 "**RNAi Molecule**" means an exogenous double-stranded oligomeric (*i.e.*, RNA or modified variants thereof) molecule.

1.293 "**Royalties**" has the meaning set forth in Section 8.4 (Royalties).

1.294 "**Royalty Rates**" means the applicable royalty rates set forth in Table 8.4 (Royalty Payments).

1.295 "**Royalty Term**" means, on a Licensed Product-by-Licensed Product and country-by-country basis, the period commencing on the First Commercial Sale of such Licensed Product in such country and expiring upon the last to occur of (a) the expiration of the last Valid Claim of the last to expire of [***], (b) the expiration of Regulatory Exclusivity for such Licensed Product in such country, and (c) [***] years after the First Commercial Sale of such Licensed Product in such country.

1.296 "**Sales Milestone Event**" has the meaning set forth in Section 8.3.2 (Sales Milestones).

1.297 "**Sales Milestone Payment**" has the meaning set forth in Section 8.3.2 (Sales Milestones).

1.298 "**Sarepta Arising Know-How**" has the meaning set forth in Section 12.1.2(b) (Sarepta).

1.299 "**Sarepta Arising LC/LP Patent Rights**" means any Sarepta Arising Patent Rights having claims Covering solely (a) [***], (b) [***], (c) [***], or (d) [***]; but expressly excluding any Sarepta Arising Patent Rights that also claim or otherwise disclose [***].

1.300 "**Sarepta Arising Patent Rights**" has the meaning set forth in Section 12.1.2(b) (Sarepta).

1.301 "**Sarepta Arising Technology**" means the Sarepta Arising Know-How and the Sarepta Arising Patent Rights.

1.302 "**Sarepta Licensed Technology**" means, collectively, the Sarepta Arising Know-How, Sarepta Arising Patent Rights, and Sarepta's interest in the Joint Arising Technology, in each case, without limiting any of Sarepta's obligations under this Agreement, that are Controlled by Sarepta or any of its Affiliates.

1.303 "**Sarepta Prosecuted Patent Rights**" has the meaning set forth in Section 12.2.1 (Sarepta's Right to Prosecute Patent Rights).

[***] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED.

1.304 "**Sarepta Records**" has the meaning set forth in Section 8.7.3 (Records and Audits).

1.305 "**[***]**" means [***].

1.306 "**Sarepta Research Activities**" has the meaning set forth in Section 2.1.2 (Non-Exclusive License Grant to Sarepta).

1.307 "**Sarepta Research Plan**" has the meaning set forth in Section 3.2.4 (Sarepta Research Activities).

1.308 "**SCA Competing Product**" has the meaning set forth in Section 2.10.1(b) (Exclusivity Covenants).

1.309 "**SCA Competitive Activities**" has the meaning set forth in Section 2.10.1(b) (Exclusivity Covenants).

1.310 "**SCA Exclusivity Period**" has the meaning set forth in Section 2.10.1(b) (Exclusivity Covenants).

1.311 "**[***]**" means [***].

1.312 "**SEC**" means the United States Securities and Exchange Commission or any successor Governmental Authority having substantially the same function.

1.313 "**Securitization Transaction**" has the meaning set forth in Section 16.1.2 (Securitization Transaction).

1.314 "**Selection Term**" has the meaning set forth in Section 3.1.3(a)(i) (Selection of Collaboration Targets).

1.315 "**Selling Party**" has the meaning set forth in Section 1.239 ("Net Sales").

1.316 "**Skeletal Muscle Target**" means, collectively, (a) any of the genetic targets set forth on **Schedule 1.316(a)** (Skeletal Muscle Targets) (the "**Exclusive SM Targets**"), and (b) any of the genetic targets set forth on **Schedule 1.316(b)** (Skeletal Muscle Targets) (the "**Non-Exclusive SM Targets**").

1.317 "**SM Competing Product**" has the meaning set forth in Section 2.10.1(b) (Exclusivity Covenants).

1.318 "**SM Competitive Activities**" has the meaning set forth in Section 2.10.1(b) (Exclusivity Covenants).

1.319 "**SM Exclusivity Period**" has the meaning set forth in Section 2.10.1(b) (Exclusivity Covenants).

1.320 "**SM Reserved Target**" has the meaning set forth in Section 3.1.3(b)(iii) (Selection of Collaboration Targets).

1.321 "**Subcontractor**" means a Third Party contractor engaged by a Party to perform certain obligations or exercise certain rights of such Party under this Agreement on a fee-for-service basis (including contract research organizations, Third Party Distributors, or CMOs).

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1.322 "**Sublicensee**" means any Third Party to whom a Party or any of its Affiliates has granted or grants a sublicense of its rights hereunder to Develop, Manufacture, Commercialize, or otherwise Exploit a Licensed Product, or any further sublicensee of such rights (regardless of the number of tiers, layers or levels of sublicenses of such rights) in accordance with Section 2.2 (Sublicensing Terms).

1.323 "**Substitution Right**" has the meaning set forth in Section 3.1.3(b)(ii) (Selection of Collaboration Targets).

1.324 "**Target**" means any of DMPK, DUX4, ATXN2, MMP7, HTT, ATXN1, ATXN3, or a Collaboration Target, in each case, individually or collectively as the context requires.

1.325 "**Target Competing Product**" has the meaning set forth in Section 2.10.1(a) (Exclusivity Covenants).

1.326 "**Target Competitive Activities**" has the meaning set forth in Section 2.10.1(a) (Exclusivity Covenants).

1.327 "**Target Exclusivity Period**" has the meaning set forth in Section 2.10.1(a) (Exclusivity Covenants).

1.328 "**Target Failure**" has the meaning set forth in Section 3.1.3(c) (Arrowhead Category 3 Development Responsibility).

1.329 "**Target Nomination Notice**" has the meaning set forth in Section 3.1.3(a)(ii) (Selection of Collaboration Targets).

1.330 "**Target Reply Notice**" has the meaning set forth in Section 3.1.3(a)(ii) (Selection of Collaboration Targets).

1.331 "**Tax**" and "**Taxation**" means any form of tax or taxation, levy, duty, charge, social security charge, contribution, or withholding of whatever nature (including any related fine, penalty, surcharge, or interest) imposed by, or payable to, any government, state or municipality, or any local, state, federal, or other fiscal, revenue, customs, or excise authority, body, or official in the Territory.

1.332 "**Technology Transfer Plan**" has the meaning set forth in Section 2.4 (Initial Technology Transfer).

1.333 "**Term**" has the meaning set forth in Section 13.1 (Term).

1.334 "**Terminated Products**" means (a) if this Agreement is terminated in its entirety, all Licensed Compounds and all Licensed Products under this Agreement, (b) if this Agreement is terminated in part with respect to a Program for the entire Territory, all Licensed Compounds and all Licensed Products that are the subject of such Program in the Territory, or (c) if this Agreement is terminated in part with respect to a Program for a Major Region, all Licensed Compounds and all Licensed Products that are the subject of such Program in such Major Region.

1.335 "**[***]**" means [***].

1.336 "**Territory**" means all of the countries of the world, and their territories and possessions.

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1.337 "**Third Party**" means any Person other than Arrowhead, Sarepta, or their respective Affiliates.

1.338 "**Third Party Distributor**" means, with respect to a country, any Third Party that purchases its requirements for Licensed Products in such country from Sarepta or its Affiliates or Sublicensees and is appointed as a distributor to distribute, market, and resell such Licensed Products in such country, even if such Third Party is granted ancillary rights to Develop, package, or obtain Regulatory Approval of such Licensed Product in order to distribute, market, or sell such Licensed Product in such country.

1.339 "**Third Party Expert**" has the meaning set forth in Section 7.5.3(a)(i) (Final Decision-Making Authority).

1.340 "**Trademark**" means any trademark, trade name, service mark, service name, brand, domain name, trade dress, logo, slogan, or other indicia of origin or ownership, including the goodwill and activities associated with each of the foregoing.

1.341 "**Transition Plan**" has the meaning set forth in Section 3.2.2(a)(i) (Transition Plans).

1.342 "**United States**" or "**U.S.**" means the United States and its territories, possessions and commonwealths.

1.343 "**Upfront Payment**" has the meaning set forth in Section 8.1 (Upfront Payment).

1.344 "**Urgent Material Trial Change**" has the meaning set forth in Section 3.1.1(c)(ii) (Material Trial Changes).

1.345 "**Valid Claim**" means (a) a claim of any issued and unexpired Patent Right whose validity, enforceability, or patentability has not been affected by any of the following: (i) irretrievable lapse, abandonment, revocation, cancellation, dedication to the public, or disclaimer; or (ii) a holding, finding, or decision of invalidity, unenforceability, or non-patentability by a court, governmental agency, national or regional patent office, or other appropriate body that has competent jurisdiction, such holding, finding, or decision being final and unappealable or unappealed within the time allowed for appeal; or (b) a pending claim of an unissued, pending patent application that has not been pending for more than [***] years from its earliest priority date, in which case it will cease to be considered a Valid Claim until the patent issues and recites said claim. For clarity, a holding, finding or decision being final and unappealable or unappealed means a holding, finding or decision from which no appeal can be or has been taken.

1.346 "**Withholding Taxes**" has the meaning set forth in Section 8.7.5(a) (Withholding Taxes).

2. LICENSE GRANTS; EXCLUSIVITY

2.1. License Grants to Sarepta.

2.1.1. **Exclusive License Grant to Sarepta**. Subject to the terms and conditions of this Agreement, on a Program-by-Program basis, Arrowhead hereby grants to Sarepta and its Affiliates, during the Term, an exclusive (even as to Arrowhead and its Affiliates, except as set forth in Section 2.5 (Arrowhead Retained Rights)), non-transferable (except in accordance with Section 16.1 (Assignment)), royalty-bearing, sublicensable (through multiple tiers, in accordance with Section 2.2 (Sublicensing Terms)) license under the

Arrowhead Technology (a) to Develop, Manufacture, perform Medical Affairs, Commercialize, and otherwise Exploit the Licensed Compounds and Licensed Products in the Field and in the Territory and (b) to perform its obligations and exercise its rights under Section 12.3 (Third Party Infringement and Defense).

2.1.2. **Non-Exclusive License Grant to Sarepta**. Subject to the terms and conditions of this Agreement, Arrowhead hereby grants to Sarepta, during the SM Exclusivity Period [***].

2.2. Sublicensing Terms.

2.2.1. Subject to this Section 2.2 (Sublicensing Terms), Sarepta and its Affiliates may grant sublicenses under Section 2.1 (License Grants to Sarepta) to any Third Party, including to any Subcontractor to the extent a sublicense of the rights granted to Sarepta hereunder is necessary for such Subcontractor to satisfy Sarepta's obligations as delegated to such Subcontractor.

2.2.2. With respect to any sublicense granted pursuant to Section 2.2.1 (Sublicensing Terms) or Section 2.3 (Performance through Subcontractors) to a Sublicensee or a Subcontractor, as the case may be:

(a) any such sublicense or subcontract agreement will be consistent with the terms of this Agreement and obligate the Sublicensee or Subcontractor to comply with the applicable terms of this Agreement;

(b) the sublicensing or subcontracting Party will remain primarily liable to the other Party for the performance of all of its obligations under, and its compliance with all provisions of, this Agreement, and for the performance of its Sublicensees and its Subcontractors, and the other Party will have the right to proceed directly against the sublicensing or subcontracting Party without any obligation to first proceed against the Sublicensees or Subcontractors;

(c) without limiting Section 2.2.2(a) (Sublicensing Terms), (i) each Sublicensee and Subcontractor, as applicable, will undertake in writing obligations of confidentiality and non-use regarding Confidential Information that are substantially the same as those undertaken by the Parties with respect to Confidential Information pursuant to Article 9 (Confidentiality and Publication), and (ii) with respect to Sarepta as the sublicensing or subcontracting Party, will use Commercially Reasonable Efforts to require that each of its Sublicensee and Subcontractor undertakes in writing to assign or exclusively license back (with the right to sublicense) to Sarepta all Arising Know-How and Arising Patent Rights (including intellectual property with respect to any Licensed Compounds and Licensed Products conceived, invented, developed or otherwise made in the course of performing any such work); and

(d) with respect to Sarepta as the sublicensing Party, within a reasonable time after execution of any sublicense agreement with a Sublicensee that grants Development or Commercialization rights in a Major Region, Sarepta will provide to Arrowhead a copy of such agreement (other than any agreement with a Subcontractor), which

agreement may be redacted to omit any terms not necessary to determining Sarepta's and such Sublicensee's obligations under this Agreement.

2.3. Performance through Subcontractors. Subject to Section 2.2.2 (Sublicensing Terms) and Section 5.4 (Manufacturing Technology Transfer), Sarepta and any of its Affiliates may perform any of its rights or obligations under this Agreement through one or more Subcontractors. Arrowhead may perform any of its obligations under this Agreement through (a) its Affiliates and (b) one or more of the Subcontractors set forth in the applicable Development Plan or Additional R&D Plan, or, subject to Sarepta's prior written approval (not to be unreasonably withheld, conditioned or delayed), any new Subcontractor that Arrowhead proposes to engage to perform Development activities under the applicable Development Plan or Additional R&D Plan. Notwithstanding the foregoing, subject to Section 2.2.2 (Sublicensing Terms), Arrowhead's Subcontractors (i) listed in **Schedule 2.3** (Arrowhead Pre-Approved Subcontractors) and already performing Research or Development activities for one or more of the Programs under this Agreement as of the Execution Date or (ii) engaged by Arrowhead to perform ancillary facility support activities that are not specific to any Licensed Compound or Licensed Product hereunder, in each case ((i) and (ii)), are deemed pre-approved by Sarepta as of the Execution Date.

2.4. Initial Technology Transfer. Within [***] days after the Effective Date, the JDC will prepare and the JSC will review, discuss, and determine whether to approve, a written technology transfer plan setting forth the Arrowhead Know-How (excluding all Arrowhead Manufacturing Know-How, which will be provided to Sarepta in accordance with Section 5.4 (Manufacturing Technology Transfer)) in existence as of the Effective Date to be transferred to Sarepta in a commercially reasonably format and the timelines for such transfer (the "**Technology Transfer Plan**"). Arrowhead will undertake all activities reasonably necessary to complete the Technology Transfer Plan, in accordance with the timelines set forth therein, at its sole cost and expense. For clarity, Arrowhead will not be required to create any documentation or data that does not already exist as of the Effective Date.

2.5. Arrowhead Retained Rights. Except as expressly granted under Section 2.1 (License Grants to Sarepta), Arrowhead hereby expressly retains, on behalf of itself and its Affiliates, all rights under the Arrowhead Technology, including the right to (a) perform (i) the Ongoing C1 Development Activities and Additional R&D Activities, (ii) the Category 2 Program Research Activities, and (iii) the Category 3 Program Research Activities, (b) Manufacture Licensed Compounds and Licensed Products in accordance with Article 5 (Manufacturing), (c) fulfill its obligations under any agreement between the Parties for Arrowhead's performance of Development activities or Manufacturing activities on behalf of Sarepta or its Affiliates or its Sublicensees for any Licensed Compounds and Licensed Products, and (d) fulfill any other obligations expressly set forth under this Agreement.

2.6. No Other Rights. Except as otherwise expressly provided in this Agreement, under no circumstances will a Party or any of its Affiliates, as a result of this Agreement, obtain any ownership interest, license, or other right in or to any Know-How, Patent Rights, or other intellectual property of the other Party, including tangible or intangible items owned, Controlled, or developed by the other Party, or provided by the other Party to the receiving Party at any time, pursuant to this Agreement. Any rights not expressly granted by a Party under this Agreement are hereby retained by such Party.

2.7. Combination Products. Notwithstanding any other provision of this Agreement, for purposes of the license grants under Section 2.1 (License Grants to Sarepta), with respect to any Licensed Product that is a Combination Product, such license will not include any Other Component Controlled by, as applicable, Arrowhead or any of its Affiliates or Sarepta or any of its Affiliates included in any such Combination Product.

2.8. License to Arrowhead. Subject to the terms and conditions of this Agreement, Sarepta hereby grants to Arrowhead and its Affiliates a non-exclusive, non-transferable (except in accordance with Section 16.1 (Assignment)), royalty-free, fully paid-up, sublicensable (to a Subcontractor in accordance with Section 2.3 (Performance through Subcontractors)), license under the Sarepta Arising Technology, solely to the extent necessary to enable Arrowhead to perform its obligations under and in accordance with the terms of this Agreement.

2.9. Third Party In-License Payments.

2.9.1. **Prior to the Effective Date**. As between the Parties, (a) except as specified in clause (b) with respect to the [***] Agreement, [***] shall be solely responsible for any license fees, milestones, royalties, and other payments, whether accruing prior to, on or following the Effective Date, under any of the Pre-Existing Third Party Agreements, and (b) solely in connection with [***], [***] shall be solely responsible [***] for all royalty payments and milestone payments of any kind owed under the [***] Agreement that are directly attributable to the Development, Manufacture, or Commercialization of any Licensed Product that is the subject of such Category 3 Program and that is also an "[***]" under the [***] Agreement (as such term is defined therein as of the Execution Date).

2.9.2. **After Effective Date**.

(a) **Program-Specific Third Party Rights**. On a Program-by-Program basis, if, in the reasonable opinion of Sarepta, rights under any Patent Rights or Know-How of a Third Party are necessary or reasonably useful for the Exploitation of any of the Licensed Compounds or Licensed Products that are the subject of such Program by Sarepta or any of its Affiliates or any of its or their Sublicensees in any country of the Territory that are or is not Platform Third Party Rights ("**Program-Specific Third Party Rights**"), then, as between the Parties, [***].

(b) **Platform Third Party Rights**.

(i) From and after the Effective Date and continuing during the Term, subject to Sarepta's rights under Section 12.5.2 (Defense), prior to Arrowhead (or any of its Affiliates) entering into an agreement with respect to any Patent Rights or Know-How of a Third Party that are or is: (A) generally applicable to making, using, or selling RNAi Molecules; (B) not specific to a Licensed Compound, a Licensed Product, or any other RNAi Molecule Directed To the Target that is the subject of a Program, or any method of manufacture or use thereof; and (C) in the reasonable opinion of Arrowhead is necessary or reasonably useful for the Exploitation of the Licensed Compounds or Licensed Products that are the subject of a Program (such Patent Rights or Know-How, a "**Platform Third Party Rights**" and such agreement, a "**Platform Third Party Agreement**"),

Arrowhead will provide written notice to Sarepta of Arrowhead's (or its Affiliate's) intent to enter into such proposed Platform Third Party Agreement, along with reasonably detailed information regarding the proposed financial terms, as well as any other material terms applicable to sublicensees under such proposed Platform Third Party Agreement and the relevant Patent Rights or Know-How owned or otherwise controlled by such Third Party that are proposed to be included as Arrowhead Technology if Sarepta elects to take a sublicense under such proposed Platform Third Party Agreement pursuant to Section 2.9.2(b)(ii) (Platform Third Party Rights). After receipt of such notice from Arrowhead with respect to any Platform Third Party Agreement, Sarepta will have the right to request discussions with Arrowhead, and, if so requested, the Parties will promptly meet and discuss such Platform Third Party Rights and Platform Third Party Agreement, including the proposed financial terms and other terms applicable to sublicensees thereunder.

(ii) Arrowhead (or its Affiliate) will use Commercially Reasonable Efforts to obtain sublicensable licenses or other rights under the relevant Platform Third Party Rights pursuant to its corresponding Platform Third Party Agreement that are sufficient to grant Sarepta a license with respect to the Licensed Compounds and Licensed Products that are the subject of the applicable Program on terms substantially consistent with the rights and licenses granted to Sarepta under the Arrowhead Technology pursuant to Section 2.1 (Licensed Grants to Sarepta); *provided* that [***]. In no event will Arrowhead enter into any Platform Third Party Agreement under which rights are not sublicensable to Sarepta in a manner that precludes Sarepta from entering into an agreement with the applicable Third Party for a grant of such Platform Third Party Rights to Exploit the Licensed Compounds and Licensed Products in the Field in the Territory.

(iii) If Arrowhead (or its Affiliate) is successful in obtaining such sublicensable licenses or other rights under the applicable Platform Third Party Agreement in accordance with Section 2.9.2(b) (Platform Third Party Rights), then (A) Sarepta will have the right, by delivery of written notice to Arrowhead, to elect to take a sublicense under such relevant Patent Rights or Know-How in-licensed by Arrowhead (or its Affiliate) under such Platform Third Party Agreement, and (B) if Sarepta makes such election, (1) [***] and (2) Sarepta agrees to comply, and will cause its Affiliates and its and their Sublicensees to comply, with any applicable obligations under such Platform Third Party Agreement that apply to Sarepta (or its Affiliates or its or their Sublicensees) as sublicensees thereunder and of which Sarepta was informed by Arrowhead in writing prior to such election by Sarepta pursuant to this Section 2.9.2(b)(ii) (Platform Third Party Rights), including [***]. If Sarepta fails to deliver such written notice to Arrowhead or otherwise declines such a sublicense, then the Platform Third Party Right subject to such Platform Third Party Agreement will not be included within the Arrowhead Technology or in any of the licenses and other rights granted to Sarepta and its Affiliates and its and their Sublicensees under this Agreement.

(iv) Nothing in this Section 2.9.2(b) (Platform Third Party Rights) restricts Sarepta's right to obtain any license or other rights in or to any Platform Third Party Right directly from any Third Party that owns or otherwise controls any Platform Third Party Right.

2.10. Exclusivity.

2.10.1. **Exclusivity Covenants**. Subject to Section 2.10.2 (Arrowhead Change of Control), except as expressly permitted under this Agreement:

(a) during [***] and on a Target-by-Target basis (the "**Target Exclusivity Period**"), Arrowhead will not, and will ensure that its Affiliates do not, independently or for or with any Third Party, Develop or Commercialize in the Territory any compound or product that is Directed To such Target [***], including through the use of Arrowhead Technology, ligand and antibody technologies, or siRNA (such compound or product, a "**Target Competing Product**" and such activities, the "**Target Competitive Activities**"), except in accordance with Section 13.7.2 (Exclusivity);

(b) during [***] (the "**SCA Exclusivity Period**"), Arrowhead will not, and will ensure that its Affiliates do not, independently or for or with any Third Party, Develop or Commercialize in the Territory any compound or product for [***], including through the use of Arrowhead Technology, including ligand and antibody technologies, or siRNA (such compound or product, a "**SCA Competing Product**" and such activities, the "**SCA Competitive Activities**"), except in accordance with Section 13.7.2 (Exclusivity);

(c) until [***] (the "**SM Exclusivity Period**"), Arrowhead will not, and will ensure that its Affiliates do not, independently or for or with any Third Party, Develop or Commercialize in the Territory any compound or product that is Directed To an Exclusive SM Target [***], including through the use of Arrowhead Technology, including ligand and antibody technologies, or siRNA (such compound or product, an "**SM Competing Product**" and such activities, the "**SM Competitive Activities**"); and

(d) during [***], Arrowhead will not, and will ensure that its Affiliates do not, independently or for or with any Third Party, Develop or Commercialize in the Territory any compound or product that is Directed To any SM Reserved Target [***], including through the use of Arrowhead Technology, ligand and antibody technologies, or siRNA.

2.10.2. **Arrowhead Change of Control**. If, during, as applicable the Target Exclusivity Period, the SCA Exclusivity Period, or the SM Exclusivity Period, Arrowhead undergoes a Change of Control and the Acquirer is (a) engaged in, respectively, Target Competitive Activities, SCA Competitive Activities, or SM Competitive Activities as of the closing of such Change of Control or (b) initiates, respectively, Target Competitive Activities, SCA Competitive Activities, or SM Competitive Activities no earlier than five years after the closing of such Change of Control, then the restrictions set forth in Section 2.10.1 (Exclusivity Covenants) will not apply to such Acquirer and such Target Competitive

Activities, SCA Competitive Activities, or SM Competitive Activities, as applicable; *provided* that (i) no Licensed Product-Specific Patent Rights, Sarepta Arising Technology, or Confidential Information of Sarepta or Confidential Information of both Parties is used by or on behalf of such Acquirer in connection with any performance of such Target Competitive Activities, SCA Competitive Activities, or SM Competitive Activities, as applicable, and (ii) such Acquirer institutes commercially reasonable technical and administrative safeguards to ensure the requirements set forth in the foregoing clause (i) are met, including by creating "firewalls" between the personnel working on the Target Competing Product, SCA Competing Product, or SM Competing Product, as applicable, and the personnel teams charged with working on any Licensed Compound or Licensed Product that is the subject of any Program hereunder having as its Target the same as that of such Target Competitive Activities, SCA Competitive Activities, or SM Competitive Activities, as applicable, or having access to data from activities performed under this Agreement or Confidential Information of Sarepta or Confidential Information of both Parties. Notwithstanding the foregoing, the foregoing clause (ii) will not apply to employees or members of the Board of Directors of Arrowhead who do not perform any day-to-day responsibilities for a Licensed Compound or a Licensed Product or, as applicable, Target Competitive Activities, SCA Competitive Activities, or SM Competitive Activities, if Arrowhead ensures that such employees and members of its Board of Directors comply with Arrowhead's obligations of confidentiality and non-use as set forth in this Agreement.

2.10.3. **Acquired Business Exception**. Notwithstanding the restrictions set forth in Section 2.10.1 (Exclusivity Covenants), if, during as applicable the Target Exclusivity Period or the SM Exclusivity Period, Arrowhead or any of its Affiliates acquires any assets or business, whether by way of merger, business combination, asset purchase, stock purchase, or otherwise (the "**Acquired Business**"), and such Acquired Business, immediately prior to such acquisition, owns, has, or includes any license or other right to any Target Competing Product, SCA Competing Product, or SM Competing Product, as applicable, that would otherwise violate Section 2.10.1 (Exclusivity Covenants), then Arrowhead will (1) notify Sarepta of such Target Competing Product, SCA Competing Product, or SM Competing Product, as applicable, in writing no later than [***] days after the consummation of such acquisition, and (2) perform one of the following acts (and specify which of the following it will perform in such notice, which decision will be final and binding on Arrowhead and its Affiliates), and in the case of all acts specified under the clauses below Arrowhead and its Affiliates also will comply with the firewalling and other requirements specified in clauses (a) and (b) of Section 2.10.2 (Arrowhead Change of Control):

(a) Arrowhead may elect to terminate the Development, Manufacture, or Commercialization of, as applicable, such Target Competing Product, SCA Competing Product, or SM Competing Product, as applicable, in which case Arrowhead and its Affiliates will cease the Development, Manufacture, and Commercialization of, as applicable, such Target Competing Product, SCA Competing Product, or SM Competing Product, as applicable, as soon as reasonably practicable and in any event within [***] days after the consummation of the acquisition of the Acquired Business, giving due consideration to ethical concerns and requirements under applicable Law and any agreements with Third Parties and notify Sarepta in writing of such completed termination; or

(b) Arrowhead may elect to divest itself (or cause its Affiliate to divest itself) of, as applicable, such Target Competing Product, SCA Competing Product, or SM Competing Product, as applicable, and notify Sarepta in writing of such completed divestiture, *provided* that such divestiture is completed within [***] months after the consummation of the acquisition of the Acquired Business.

2.10.4. **Acknowledgment**. Arrowhead acknowledges and agrees that (a) this Section 2.10 (Exclusivity) has been negotiated by the Parties, (b) the geographical and time limitations on activities set forth in Section 2.10 (Exclusivity) are reasonable, valid, and necessary in light of the Parties' circumstances and necessary for the adequate protection of the Research, Development, Manufacturing, Commercialization, and other Exploitation of the Licensed Compounds and Licensed Products, and (c) Sarepta would not have entered into this Agreement without the protection afforded it by Section 2.10 (Exclusivity). If, notwithstanding the foregoing, a court of competent jurisdiction determines that the restrictions set forth in Section 2.10 (Exclusivity) are too broad or otherwise unreasonable (for example, due to a change in circumstance) under applicable Law, including with respect to duration, geographic scope, or space, then the court is hereby requested and authorized by the Parties to, and if the court cannot do so, then the Parties will, revise Section 2.10 (Exclusivity) to include the maximum restrictions allowable under applicable Law.

3. RESEARCH AND DEVELOPMENT

3.1. Arrowhead Research and Development Activities.

3.1.1. **Category 1 Program Development**.

(a) **Arrowhead Category 1 Program Development Responsibility**. Subject to Section 3.4 (Arrowhead Development Costs Reimbursement), on a Category 1 Program-by-Category 1 Program basis, Arrowhead will be responsible for conducting and completing the Ongoing Development Trials and all Ongoing C1 Development Activities, which activities Arrowhead will perform in accordance with the applicable corresponding Category 1 Development Plan for such Category 1 Program; *provided* that Sarepta may assume responsibility and control of the Ongoing Development Trials and associated Ongoing C1 Development Activities for any Category 1 Program (i) as requested by Sarepta and approved by the JSC (A) at any time for each Category 1 Program other than the DM1 Program, and (B) for the DM1 Program, solely after the earlier of (1) the date of occurrence of the DM1 Second Development Milestone Event and (2) the date on which Sarepta, in its sole discretion, pays Arrowhead the remaining amount owed to Arrowhead under Section 8.3.1(a) (DM1 Program Development Milestones) as of such date as if both the DM1 First Development Milestone Event and the DM1 Second Development Milestone Event had been achieved or (ii) at any time, in the exercise of Sarepta's step-in right in accordance with Section 3.1.1(b) (Sarepta Category 1 Development Step-In Right) (such assumed Clinical Trials and activities, the "**Assumed C1 Program Development Activities**"). To the extent not set forth in the Transition Plan for a given Category 1 Program with respect to which Sarepta is to perform any Assumed C1 Program Development Activities, Arrowhead will provide to Sarepta copies of all Arrowhead Know-How that is necessary or, in the

Parties' mutual good faith opinion, reasonably useful for the performance of such Assumed C1 Program Development Activities. Arrowhead will not perform any Research or other Development activities for any Licensed C1 Compound or Licensed C1 Product of any Category 1 Program other than in accordance with the applicable Category 1 Development Plan for such Category 1 Program or, if any, the applicable Additional R&D Plan for such Category 1 Program. For clarity, if Sarepta makes the payment contemplated in the foregoing clause (a)(i)(B)(2), then, upon Arrowhead's receipt of such payment, Sarepta will have no further obligations under Section 8.3.1(a) (DM1 Program Development Milestones).

(b) **Sarepta Category 1 Development Step-In Right**. On a Category 1 Program-by-Category 1 Program basis, if Sarepta, acting in good faith, determines that Arrowhead has failed to perform, one or more of its Ongoing C1 Development Activities under and in accordance with the applicable corresponding Category 1 Development Plan for such Category 1 Program, then Sarepta will provide Arrowhead with written notice regarding such failure to perform, and upon receipt of such notice Arrowhead will have a period of [***] days to perform (or re-perform) the applicable Ongoing C1 Development Activities, or a longer period to the extent determined by Sarepta in its reasonable discretion following consultation in good faith with Arrowhead to allow such performance or re-performance of the relevant activity that gave rise to the failure, assuming the use of diligent efforts by Arrowhead ("**Ongoing C1 Development Activities Cure Period**"). If (i) Arrowhead has not completed performance of such Ongoing C1 Development Activities in accordance with the applicable Category 1 Development Plan upon the expiration of the applicable Ongoing C1 Development Activities Cure Period or (ii) Arrowhead otherwise notifies Sarepta in writing that Arrowhead anticipates that it will be unable to perform or complete any Ongoing C1 Development Activities, as applicable, in accordance with this Section 3.1.1 (Category 1 Program Development) and Section 3.3.1 (Arrowhead Development Diligence Obligations), then, in each case ((i) and (ii)), Sarepta may, upon written notice to Arrowhead, assume such Ongoing C1 Development Activities that are subject to such default by Arrowhead. Except as otherwise set forth in Section 7.5.3(b) (Final Decision-Making Authority) or Section 7.5.3(c) (Final Decision-Making Authority), in each case, with respect to a DM1 Development Milestone MTC, Sarepta will have sole control over and decision-making authority with respect to such Assumed C1 Program Development Activities, which Sarepta will perform at its cost and expense.

(c) **Category 1 Development Plans; Material Trial Changes**.

(i) **Category 1 Development Plans**. Subject to Section 3.4 (Arrowhead Development Costs Reimbursement), on a Category 1 Program-by-Category 1 Program basis, Arrowhead will conduct all activities in furtherance of the Ongoing C1 Development Activities for such Category 1 Program in accordance with a written plan to be prepared by the JDC and submitted to the JSC to review, discuss, and determine whether to approve as promptly as reasonably practicable after the Effective Date (and in any event no later than [***] days after the Effective Date) (as such plan may be updated thereafter in accordance with this Agreement, a

"**Category 1 Development Plan**"). Each Category 1 Development Plan will include (and any subsequent update thereof will include) (A) the planned Ongoing C1 Development Activities to be conducted by or on behalf of Arrowhead through completion of the Ongoing Development Trials for such Category 1 Program and the timelines of such activities, (B) the Manufacturing activities (including all CMC Activities) to be conducted by or on behalf of Arrowhead in support of the Ongoing C1 Development Activities (if any), (C) the Sarepta Personnel that may assist Arrowhead in the performance of any of the activities described in the foregoing clauses (A) and (B) and a summary of the activities with which such Sarepta Personnel may assist, and (D) a budget that sets forth all FTE Costs and Out-of-Pocket Costs to be incurred by or on behalf of Arrowhead in the performance of the Ongoing C1 Development Activities (for each Category 1 Program, a "**Category 1 Development Budget**"). Either Party, through the JDC, may propose updates (other than any Material Trial Change, which are addressed in Section 3.1.1(c)(ii) (Material Trial Changes)) to any Category 1 Development Plan (including the Category 1 Development Budget set forth therein) and the JSC will review, discuss, and determine whether to approve each such update, including updates to the Category 1 Development Budget therein.

(ii) **Material Trial Changes**. No Material Trial Change to an Ongoing Clinical Trial will be implemented without the approval of the Parties through the JSC. In the event a Party wishes to propose a Material Trial Change to an Ongoing Clinical Trial, the Parties, through the JDC, will discuss in good faith such Material Trial Change, including reviewing and discussing all material information relevant to such proposed Material Trial Change, and following such discussion, the JSC will determine whether to approve the implementation of such Material Trial Change and the JSC will update the applicable Category 1 Development Plan (including the Category 1 Development Budget) to include such approved Material Trial Change (with no further decision-making authority over such update). Notwithstanding the preceding sentence, and subject to Section 4.4 (Pharmacovigilance Agreement), (A) no such prior written approval by Sarepta through the JSC will be needed for, and instead Arrowhead will provide Sarepta prompt written notice of (including all material information relevant to such Material Trial Change) and will discuss promptly with Sarepta, the implementation of a Material Trial Change reasonably necessary for patient safety or required by a Regulatory Authority (an "**Urgent Material Trial Change**") and the JSC will update the applicable Category 1 Development Plan (including the Category 1 Development Budget) to include such Urgent Material Trial Change (with no further decision-making authority over such update); *provided* that if Sarepta reasonably believes that Arrowhead should undertake a particular course of action in relation to a proposed Urgent Material Trial Change to reduce risks to patient safety, then Arrowhead will consider in good faith such course of action as part of such Urgent Material Trial Change, and (B) except as set forth in the foregoing clause (A), Sarepta will provide its decision as to whether to consent to such

Material Trial Change directly to Arrowhead and not through the JSC (which consent may be via email or other electronic communication) no later than five Business Days (or such longer period as reasonably agreed in writing by the Parties) after the date of Arrowhead's written request (including all material information relevant to such Material Trial Change for such approval) for approval of (1) any change described in clause (d) of the definition of "**Material Trial Change**" and (2) any other Material Trial Change identified by Arrowhead acting in good faith as urgent.

3.1.2. **Category 2 Program Research and Development**.

(a) **Arrowhead Category 2 Program Development Responsibility**. On a Category 2 Program-by-Category 2 Program basis, Arrowhead will be responsible for conducting and completing the Category 2 Program Research Activities in accordance with the applicable Category 2 Development Plan for such Category 2 Program to deliver to Sarepta a Category 2 CTA Ready Package therefor. Arrowhead will not perform any Research or other non-clinical or clinical Development activities with respect to a Category 2 Program other than in accordance with its applicable Category 2 Development Plan or, if any, the applicable Additional R&D Plan for such Category 2 Program.

(b) **Category 2 Development Plans**. Arrowhead will conduct the Category 2 Program Research Activities for each Category 2 Program in accordance with a written plan to be prepared by the JDC and submitted to the JSC to review, discuss, and determine whether to approve as promptly as reasonably practicable after the Effective Date (and in any event no later than [***] days after the Effective Date) (as such plan may be updated thereafter in accordance with this Agreement, a "**Category 2 Development Plan**"). Each Category 2 Development Plan for a Category 2 Program will include (and any subsequent update thereof will include) (i) the planned Research and pre-clinical Development activities to be conducted by or on behalf of Arrowhead and the timelines of such activities, (ii) the Manufacturing activities (including all CMC Activities) to be conducted by or on behalf of Arrowhead in support of the activities described in the foregoing clause (i) (if any), (iii) the Sarepta Personnel that may assist Arrowhead in the performance of any of the activities described in the foregoing clauses (i) and (ii) and a summary of the activities with which such Sarepta Personnel may assist, and (iv) the transfer to Sarepta of the data, results, and other material Know-How, including the chemical structures and sequences of a lead Licensed C2 Product that is the subject of such Category 2 Program (but excluding all Arrowhead Manufacturing Know-How, which will be provided to Sarepta in accordance with Section 5.4 (Manufacturing Technology Transfer)), that are (A) generated in the performance of activities of clause (i) and (B) necessary or, in the Parties' mutual good faith opinion, reasonably useful for Sarepta to file a CTA for such Licensed C2 Product to commence a Phase I Clinical Trial, but in any event including all information set forth on and (if specified therein) in the format specified in **Schedule 3.1.2** (CTA Ready Package Form) (the "**Category 2 CTA Ready Data**") (collectively, the data, results and Know-How of clause (iv) the "**Category 2 CTA Ready Package**", and together with the activities of clause (i) and clause (ii), the "**Category 2 Program Research Activities**"). Either Party, through the JDC, may

propose updates to a Category 2 Development Plan and the JSC will review, discuss, and determine whether to approve each such update. The Parties agree that, as of the Execution Date, the anticipated lead Licensed C2 Product for the HTT Program is ARO-HTT.

(c) **Sarepta Category 2 CTA Ready Package Acceptance**. Following its receipt of a Category 2 CTA Ready Package with respect to a Category 2 Program, Sarepta will have [***] days to (i) review the contents of such Category 2 CTA Ready Package and (ii) ask any reasonable questions regarding such Category 2 CTA Ready Package, which Arrowhead will promptly answer. If Sarepta, acting in good faith, determines that such Category 2 CTA Ready Package is incomplete, as compared to the Category 2 CTA Ready Data specified on **Schedule 3.1.2** (CTA Ready Package Form) to be included in such Category 2 CTA Ready Package or that Arrowhead has not otherwise provided Sarepta with any Arrowhead Know-How that is generated in the performance of activities under the applicable Category 2 Development Plan that is necessary or, in the Parties' mutual good faith opinion, reasonably useful for Sarepta to file a CTA for one or more Licensed C2 Products that are the subject of such Category 2 Program, then Sarepta will promptly notify Arrowhead of such deficiency and identify in reasonable detail in such notice the missing Category 2 CTA Ready Data or other Arrowhead Know-How. Unless Arrowhead disputes in good faith such deficiency, in which case such dispute will be referred to the JSC for resolution, Arrowhead shall promptly deliver to Sarepta such previously missing Category 2 CTA Ready Data. Sarepta will be deemed to have accepted the Category 2 CTA Ready Package as complete and final upon the date of expiration of such [***] day period; provided, however, that, if applicable, such period will be extended by the number of days that are necessary such that Sarepta has [***] days following its receipt of all identified missing Category 2 CTA Ready Data in response to its notice of deficiency, and the Category 2 CTA Ready Package will be deemed accepted by Sarepta upon the expiration of such supplemental time period.

3.1.3. **Category 3 Program Research and Development**.

(a) **Selection of Collaboration Targets**.

(i) Sarepta will have the right to select up to six targets from among the CNS Targets, Skeletal Muscle Targets, and Cardiomyocyte Targets at any time until [***] (the "**Selection Term**"); *provided* that [***].

(ii) At any time during the Selection Term, Sarepta may send a written notice to Arrowhead identifying a proposed target in accordance with the selection limitations set forth in Section 3.1.3(a)(i) (a "**Target Nomination Notice**"). [***] If the Target Nomination Notice nominates a Non-Exclusive SM Target, CNS Target (other than [***]), or a Cardiomyocyte Target, then Arrowhead will have [***] days after receiving such Target Nomination Notice to provide written notice to Sarepta of whether Arrowhead accepts or rejects such nominated Non-Exclusive SM Target, CNS Target, or Cardiomyocyte Target (a "**Target Reply Notice**"). Arrowhead may reject such nominated Non-Exclusive

SM Target, CNS Target, or Cardiomyocyte Target only if: [***]. If Arrowhead provides Sarepta a Target Reply Notice rejecting such nominated Non-Exclusive SM Target, CNS Target, or Cardiomyocyte Target on the basis of the foregoing clause (A) or clause (B), then such Target Nomination Notice will be voided and such nominated target Non-Exclusive SM Target, CNS Target, or Cardiomyocyte Target will not become the target of a Category 3 Program under this Agreement for any purposes, including Section 2.10.1 (Exclusivity Covenants) and Section 3.3.1 (Arrowhead Development Diligence Obligations). If Arrowhead provides Sarepta a Target Reply Notice accepting such nominated Non-Exclusive SM Target, CNS Target, or Cardiomyocyte Target, then such nominated Non-Exclusive SM Target, CNS Target, or Cardiomyocyte Target will become the target of a Category 3 Program under this Agreement. Each target accepted by Arrowhead pursuant to this Section 3.1.3(a) (Selection of Collaboration Targets) will be deemed a "**Collaboration Target**" under this Agreement. Subject to Section 3.1.3(b) (Sarepta Collaboration Target Substitution Right), Sarepta may send Target Nomination Notices to Arrowhead during the Selection Term until six targets have been accepted by Arrowhead as Collaboration Targets. If, during the Term, Arrowhead enters into a collaboration, license, or other arrangement with a Third Party that includes Arrowhead using its platforms and other proprietary technology to Research, Develop, Manufacture, Commercialize, or otherwise Exploit compounds and products Directed To Non-Exclusive SM Targets, CNS Targets, or Cardiomyocyte Targets, then the Parties agree to negotiate in good faith and execute an amendment to this Agreement to provide for a gatekeeping process to govern Sarepta's selection of Non-Exclusive SM Targets, CNS Targets, or Cardiomyocyte Targets to be pursued as Collaboration Targets under the terms and conditions of this Agreement.

(iii) If, during the Selection Term, Arrowhead provides Sarepta with a Target Reply Notice rejecting, as permitted under Section 3.1.3(a) (Selection of Collaboration Targets), a target proposed in a Target Nomination Notice delivered by Sarepta, then Sarepta will have the right (but not the obligation) to provide Arrowhead a Target Nomination Notice nominating an alternate nominated target at any time prior to the end of the Selection Term, which alternate nominated target will be subject to the selection process set forth under this Section 3.1.3(a) (Selection of Collaboration Targets).

(b) **Sarepta Collaboration Target Substitution Right**.

(i) If, during the Selection Term, Arrowhead notifies Sarepta in writing of a Target Failure with respect to an ongoing Category 3 Program as contemplated under Section 3.1.3(c) (Arrowhead Category 3 Development Responsibility), then Sarepta will have the right (but not the obligation) to provide Arrowhead a Target Nomination Notice nominating an alternate nominated target at any time prior to the end of the Selection

Term, which alternate nominated target will be subject to the selection process of Section 3.1.3(a) (Selection of Collaboration Targets).

(ii) Any alternate nominated target pursuant to Section 3.1.3(b)(i) (Sarepta Collaboration Target Substitution Right) will be a "**Replacement Target**" and, if accepted by Arrowhead in accordance with Section 3.1.3(a)(ii) (Selection of Collaboration Targets) or 3.1.3(b)(iii) (Sarepta Collaboration Target Substitution Right), as applicable, will be deemed a Collaboration Target (each such occurrence of a Replacement Target accepted by Arrowhead, a "**Substitution Right**"); [***].

(iii) At the end of the Selection Term and provided that Sarepta has not already exercised its [***] Substitution Rights, Sarepta will identify in writing to Arrowhead [***] targets from amongst the Exclusive SM Targets to be reserved for Sarepta's selection as an alternate target for a Category 3 Program in case of a Target Failure with respect to an ongoing Category 3 Program as contemplated under Section 3.1.3(c) (Arrowhead Category 3 Development Responsibility) during the [***] year period after the expiration of the Selection Term (each, a "**SM Reserved Target**").

(iv) During the [***] year period after the expiration of the Selection Term, if Arrowhead notifies Sarepta in writing of a Target Failure with respect to an ongoing Category 3 Program as contemplated under Section 3.1.3(c) (Arrowhead Category 3 Development Responsibility), then Sarepta will have the right (but not the obligation) to (A) submit to Arrowhead a Target Nomination Notice nominating an alternate nominated target, which target will be subject to the selection process of Section 3.1.3(a) (Selection of Collaboration Targets), or (B) provide written notice to Arrowhead identifying the SM Reserved Target to be substituted as a Collaboration Target hereunder, in either case ((A) or (B)), within [***] days after, as applicable, the date that Sarepta is notified by Arrowhead of the Target Failure.

(v) Neither Sarepta nor Arrowhead will have any further rights with respect to (A) any Collaboration Target for which there was a Target Failure, or (B) all SM Reserved Targets, if any, that have not become Collaboration Targets on the date that is [***] years after the expiration of the Selection Term, and, in each case ((A) and (B)), either Party will be free to pursue any compounds and products Directed To such targets outside of this Agreement.

(c) **Arrowhead Category 3 Development Responsibility**. During the Collaboration Term, on a Category 3 Program-by-Category 3 Program basis, Arrowhead will be responsible, at its sole cost and expense, for conducting and completing the Category 3 Program Research Activities for the Collaboration Target of such Category 3 Program in accordance with the applicable Category 3 Development Plan for such Category 3 Program with the aim of delivering to Sarepta a Category 3 CTA Ready Package therefor. Arrowhead will not perform any Research or other non-clinical or clinical Development activities for any Collaboration Target other

than in accordance with the applicable Category 3 Development Plan or, if any, the applicable Additional R&D Plan for such Category 3 Program. If Arrowhead, acting in good faith, determines that the data and results generated in its performance of the Category 3 Program Research Activities for a Category 3 Program show achievement of the [***] specified for such Category 3 Program in the applicable Category 3 Development Plan, then Arrowhead will promptly notify Sarepta in writing of such determination, and each Party's rights and obligations with respect to such Category 3 Program and its corresponding Collaboration Target will terminate (a "**Target Failure**"), including pursuant to Section 2.10 (Exclusivity Covenants) and Section 3.3.1 (Arrowhead Development Diligence Obligations); *provided* that if Sarepta, acting in good faith, disputes the achievement of the [***] with respect to any Category 3 Program and its termination under this Agreement, then such dispute will be referred to the JSC for resolution.

(d) **Category 3 Development Plans**. Arrowhead will conduct the Category 3 Program Research Activities for each Category 3 Program in accordance with a written plan prepared by the JDC and submitted to the JSC to review, discuss, and determine whether to approve as promptly as reasonably practicable after the date of Arrowhead's Target Reply Notice accepting the target nominated in Sarepta's corresponding Target Nomination Notice (and in any event no later than [***] days after the date of such Target Reply Notice) (as such plan may be updated thereafter in accordance with this Agreement, a "**Category 3 Development Plan**"). The Category 3 Development Plan for a Category 3 Program will include (and any subsequent update thereof will include) (i) the planned Research and pre-clinical Development activities to be conducted by or on behalf of Arrowhead and the timelines of such activities, (ii) the Manufacturing activities (including all CMC Activities) to be conducted by or on behalf of Arrowhead in support of the activities described in the foregoing clause (i) (if any), (iii) the Sarepta Personnel that may assist Arrowhead in the performance of any of the activities described in the foregoing clauses (i) and (ii) and a summary of the activities with which such Sarepta Personnel may assist, and (iv) the transfer to Sarepta of the data, results and other material Know-How, including the chemical structures and sequences of a lead Licensed C3 Product under such Category 3 Program (but excluding all Arrowhead Manufacturing Know-How which will be provided to Sarepta in accordance with Section 5.4 (Manufacturing Technology Transfer)), that are (A) generated in the performance of activities of clause (i) and (B) necessary or, in the Parties' mutual good faith opinion, reasonably useful for Sarepta to file a CTA for such Licensed C3 Product to commence a Phase I Clinical Trial, but in any event including all information set forth on and (if specified therein) in the format specified in **Schedule 3.1.2** (CTA Ready Package Form) (the "**Category 3 CTA Ready Data**") (collectively, the data, results and Know-How of clause (iv) the "**Category 3 CTA Ready Package**", and together with the activities of clause (i) and clause (ii), the "**Category 3 Program Research Activities**"). Either Party, through the JDC, may propose updates to any Category 3 Development Plan and the JSC will review, discuss, and determine whether to approve each such update.

(e) **Sarepta Category 3 CTA Ready Package Acceptance**. Following its receipt of a Category 3 CTA Ready Package with respect to a Category 3 Program, Sarepta

will have [***] days to (i) review the contents of such Category 3 CTA Ready Package and (ii) ask any reasonable questions regarding such Category 3 CTA Ready Package, which Arrowhead will promptly answer. If Sarepta, acting in good faith, determines that such Category 3 CTA Ready Package is incomplete, as compared to the Category 3 CTA Ready Data specified on **Schedule 3.1.2** (CTA Ready Package Form) to be included in such Category 3 CTA Ready Package or that Arrowhead has not otherwise provided Sarepta with any Arrowhead Know-How that is generated in the performance of activities under the applicable Category 3 Development Plan that is necessary or, in the Parties' mutual good faith opinion, reasonably useful for Sarepta to file a CTA for one or more Licensed C3 Products that are the subject of such Category 3 Program, Sarepta will promptly notify Arrowhead of such deficiency and identity in reasonable detail in such notice the missing Category 3 CTA Ready Data or other Arrowhead Know-How. Unless Arrowhead disputes in good faith such deficiency, in which case such dispute will be referred to the JDC for resolution, Arrowhead shall promptly deliver to Sarepta such previously missing Category 3 CTA Ready Data. Sarepta will be deemed to have accepted the Category 3 CTA Ready Package as complete and final upon the date of expiration of such [***] day period; provided, however, that, if applicable, such period will be extended by the number of days that are necessary to ensure that Sarepta has [***] days following its receipt of identified missing Category 3 CTA Ready Data in response to its notice of deficiency, and the Category 3 CTA Ready Package will be deemed accepted by Sarepta upon the expiration of such supplemental time period.

3.1.4. **Additional R&D Responsibilities**. During the Term, subject to Section 3.4 (Arrowhead Development Costs Reimbursement), on a Program-by-Program basis, Sarepta may request that Arrowhead perform certain (a) Research or other non-clinical Development activities for the Licensed Compounds and Licensed Products that are the subject of such Program or (b) CMC Activities, in each case ((a) and (b)), as may be reasonably necessary to support the filing of an IND with the FDA for the Licensed Compounds and Licensed Product that are the subject of such Program ("**Additional R&D Activities**"). Upon such request, the JDC will prepare a written plan that sets forth such Additional R&D Activities for the applicable Program (for each Program, as such plan may be amended in accordance with this Agreement, an "**Additional R&D Plan**") and submit such proposed Additional R&D Plan to the JSC to review, discuss, and determine whether to approve. Each Additional R&D Plan will include (and any subsequent update thereof will include) (a) the planned Additional R&D Activities to be conducted by or on behalf of Arrowhead and the timelines of such Additional R&D Activities, (b) the Manufacturing activities to be conducted by or on behalf of Arrowhead in support of the activities described in the foregoing clauses (a) or (b) (if any), (c) the Sarepta Personnel that may assist Arrowhead with any of the activities described in the foregoing clauses (a) and (b) and a summary of the activities with which such Sarepta Personnel may assist, and (d) a budget that sets forth all FTE Costs and Out-of-Pocket Costs to be incurred by or on behalf of Arrowhead in the performance of such Additional R&D Activities under such plan (for each Program, an "**Additional R&D Budget**"). Either Party may propose (through the JSC) updates to any Additional R&D Plan (including the Additional R&D Budget set forth therein) and the JSC will review, discuss, and determine whether to approve each such update to any Additional R&D Plan, including updates to the Additional R&D Budget therein.

3.2. Sarepta Development and Medical Affairs Activities.

3.2.1. **Sarepta Development and Medical Affairs Responsibility**.

(a) For each Category 1 Program, from and after the earlier of (i) the later of the date of Arrowhead's completion of the Ongoing C1 Development Activities and, if applicable, of any Additional R&D Activities for such Category 1 Program and (ii) the date of Sarepta's assumption of the Assumed C1 Program Development Activities for such Category 1 Program, except as otherwise set forth in Section 8.3.1(d) (DM1 First Development Milestone Event) with [***], Sarepta will have sole control over and decision-making authority for the Development of, and performance of Medical Affairs for, all Licensed C1 Compounds and Licensed C1 Products that are the subject of such Category 1 Program, including preparing Clinical Trial designs and protocols (other than with respect to a [***]), sponsoring Clinical Trials, engaging CROs, and managing activities at Clinical Trial sites.

(b) For each Category 2 Program, from and after the CTA Ready Package Acceptance Date for such Category 2 Program, Sarepta will have sole control over and decision-making authority for the Development of, and performance of Medical Affairs for, all Licensed C2 Compounds and Licensed C2 Products that are the subject of such Category 2 Program, including preparing Clinical Trial designs and protocols, sponsoring Clinical Trials, engaging CROs, and managing activities at Clinical Trial sites.

(c) For each Category 3 Program, from and after the CTA Ready Package Acceptance Date for such Category 3 Program, Sarepta will have sole control over and decision-making authority for the Development of, and performance of Medical Affairs for, all Licensed C3 Compounds and Licensed C3 Products that are the subject of such Category 3 Program, including preparing Clinical Trial designs and protocols, sponsoring Clinical Trials, engaging CROs, performing all Medical Affairs, and managing activities at Clinical Trial sites.

3.2.2. **Transition of Category 1 Program Development Activities**.

(a) **Transition Plans**.

(i) For each Category 1 Program, subject to Section 4.2 (Assignment of Regulatory Submissions), within [***] days following the Effective Date, the JDC will prepare and submit to the JSC to review, discuss, and determine whether to approve a written plan for each Category 1 Program setting forth the activities to be undertaken and the copies of Arrowhead Know-How to be transferred to effectuate the transition from Arrowhead to Sarepta of (A) Development activities following Arrowhead's completion of all Ongoing Development Trial(s) and Ongoing C1 Development Activities set forth in the Development Plan for such Category 1 Program or (B) any Assumed C1 Program Development Activities, in each case, for such Category 1 Program (for each Category 1 Program, a "**Transition Plan**"); *provided*, *however*, that the JDC will prepare, and submit to the JSC to review, discuss, and determine whether

to approve, the Transition Plan for the DM1 Program promptly after the earliest date on which: (1) the DM1 Second Development Milestone Event has been achieved, (2) Sarepta assumes Assumed C1 Program Development Activities with respect to the DM1 Program, or (3) [***].

(ii) With respect to any Assumed C1 Program Development Activities for a Category 1 Program [***], upon Sarepta's request, the JDC will prepare, and submit to the JSC to review, discuss, and determine whether to approve, an update to the applicable Transition Plan for such Category 1 Program to contemplate an earlier transition to Sarepta of the Assumed C1 Program Development Activities.

(iii) The JDC will coordinate the transition of all Development activities for each Category 1 Program in accordance with its applicable Transition Plan, and each Party will use reasonable efforts to perform the activities assigned to it under such Transition Plan. Sarepta will be solely responsible for all costs and expenses it incurs in connection with the performance of its activities under the applicable Transition Plan. As part of such transition, Arrowhead, at its cost and expense (except as set forth in Section 3.2.2(b) (Transition Assistance)), will promptly disclose to Sarepta all Arrowhead Know-How and any Clinical Trial Regulatory Submissions, INDs, MAAs, and other Regulatory Approvals or Regulatory Submissions related to a given Program set forth in the applicable Transition Plan (including the timelines for such disclosures to be made) and not previously transferred to Sarepta in accordance with Section 2.4 (Initial Technology Transfer) and subject to Section 4.2 (Assignment of Regulatory Submissions), excluding all Arrowhead Manufacturing Know-How that is not listed in the applicable Transition Plan, which will be provided to Sarepta in accordance with Section 5.4 (Manufacturing Technology Transfer). For clarity, in connection with the transition under this Section 3.2.2 (Transition of Category 1 Program Development Activities) for each Category 1 Program, Arrowhead will not be required to (i) except as reasonably requested by Sarepta and at Sarepta's sole cost and expense, create any documents that do not already exist as of the completion of the Ongoing Development Trial(s) and Ongoing C1 Development Activities set forth in the applicable Category 1 Development Plan for a Category 1 Program; or (ii) assign or otherwise transfer to Sarepta any master agreements to which Arrowhead or any of its Affiliates is a party that could relate to the Exploitation of compounds or products other than the Licensed C1 Compounds or Licensed C1 Products that are the subject of the Category 1 Program being transitioned to Sarepta (regardless of whether such master agreement relates to any such compound or product at such time), *provided* that, to the extent related to such compound or product, Arrowhead will provide to Sarepta a copy of such master agreement, which may be redacted to preserve Third Party confidentiality. After the Effective Date, unless otherwise agreed by Sarepta in advance, Arrowhead will not enter into any agreement with a Third Party that relates solely to a Category 1 Program (or amend any such agreement existing as of the Effective Date) pursuant to which Arrowhead

would be liable for any payment in connection with any assignment thereof to Sarepta or the assignment of which to Sarepta would require consent from such Third Party.

(b) **Transition Assistance**. Upon Sarepta's reasonable request and for a period not to exceed [***] days following completion of the activities and document transfers contemplated under the applicable Transition Plan for a Category 1 Program, Arrowhead will provide such assistance, including making its Personnel reasonably available to Sarepta during normal business hours, as is reasonably necessary to support the transition of Development and Medical Affairs activities to Sarepta for such Category 1 Program in accordance with such Transition Plan. Sarepta will reimburse Arrowhead for (a) any reasonable Out-of-Pocket Costs incurred in providing such assistance and (b) Arrowhead's reasonable costs, at the FTE Rate, incurred in connection with its Personnel's participation in any such assistance activities, including working group meetings or any other one-on-one meetings with Personnel of Sarepta or its designee, in each case ((a) and (b)), solely to the extent such Out-of-Pocket Costs and FTE Costs are set forth in a budget agreed to by the Parties in writing or through the JSC before Arrowhead undertakes the applicable assistance activities.

3.2.3. **Transition of Category 2 Program and Category 3 Research & Development Activities**.

(a) For each Category 2 Program and each Category 3 Program, Arrowhead will provide to Sarepta, at Arrowhead's cost and expense, promptly following the applicable CTA Ready Package Acceptance Date for, respectively, such Category 2 Program and such Category 3 Program, any Arrowhead Know-How (excluding Arrowhead Manufacturing Know-How to be provided to Sarepta in accordance with Section 5.4 (Manufacturing Technology Transfer)) *not* previously disclosed to Sarepta either (i) pursuant to Section 2.4 (Initial Technology Transfer) or (ii) as part of, respectively, the Category 2 CTA Ready Package for such Category 2 Program or the Category 3 CTA Ready Package for such Category 3 Program (on a Program-by-Program basis, the date of completion of such supplemental technology transfer for such Program, the "**Supplemental Technology Transfer Date**"). For clarity, Arrowhead will not be required to create any documentation or data with respect to, as applicable, a Category 2 Program or Category 3 Program that does not already exist as of immediately prior to its applicable Supplemental Technology Transfer Date.

(b) Upon Sarepta's reasonable request and for a period not to exceed [***] days following the Supplemental Technology Transfer Date for, as applicable, a Category 2 Program or Category 3 Program, Arrowhead will provide such assistance, including making its Personnel reasonably available to Sarepta during normal business hours, as is reasonably necessary to support the transition of Development and Medical Affairs activities to Sarepta for such Category 2 Program or Category 3 Program. Sarepta will reimburse Arrowhead for (i) any reasonable Out-of-Pocket Costs incurred in providing such assistance and (ii) Arrowhead's reasonable costs, at the FTE Rate, incurred in connection with its Personnel's participation in any such assistance activities, including working

group meetings or any other one-on-one meetings with Personnel of Sarepta or its designee, in each case ((i) and (ii)), solely to the extent such Out-of-Pocket Costs and FTE Costs are set forth in a budget agreed to by the Parties in writing or through the JSC before Arrowhead undertakes the applicable assistance activities.

3.2.4. [***]

3.3. Development Diligence Obligations.

3.3.1. **Arrowhead Development Diligence Obligations**.

(a) Subject to Section 3.4 (Arrowhead Development Costs Reimbursement), for each Category 1 Program, Arrowhead will use Commercially Reasonable Efforts to (i) conduct the Ongoing Development Trials and all Ongoing C1 Development Activities in accordance with the applicable Category 1 Development Plan (including the timelines set forth therein), as may be amended by the Parties through the JSC, and (ii) conduct any Additional R&D Activities in accordance with the applicable Additional R&D Plan approved by the JSC (including the timelines set forth therein), as may be amended by the Parties through the JSC.

(b) For each Category 2 Program, Arrowhead will use Commercially Reasonable Efforts to (i) conduct the Category 2 Program Research Activities in accordance with the applicable Category 2 Development Plan (including the timelines set forth therein), as may be amended by the Parties through the JSC, and (ii) conduct any Additional R&D Activities in accordance with the applicable Additional R&D Plan approved by the JSC (including the timelines set forth therein), as may be amended by the Parties through the JSC.

(c) For each Category 3 Program, Arrowhead will use Commercially Reasonable Efforts to (i) conduct the Category 3 Program Research Activities in accordance with the applicable Category 3 Development Plan (including the timelines set forth therein), as may be amended by the Parties through the JSC, and (ii) conduct any Additional R&D Activities in accordance with the applicable Additional R&D Plan approved by the JSC (including the timelines set forth therein), as may be amended by the Parties through the JSC. For clarity, and in relation to a specific Category 3 Program, the preceding sentence does not [***].

3.3.2. **Sarepta Development Diligence Obligations**.

(a) On a Program-by-Program basis, upon (i) the date that the Parties complete all activities under the Transition Plan with respect to a Category 1 Program as set forth in Section 3.2.2(a) (Transition Plans) or (ii) the Supplemental Technology Transfer Date with respect to a Category 2 Program or Category 3 Program, in each case ((i) and (ii)), Sarepta, either itself or through its Affiliates or Sublicensees, will use Commercially Reasonable Efforts to Develop and seek Regulatory Approval for at least one Licensed Product that is the subject of the applicable Program in each of (i) the United States, (ii) any three Major European Markets, and (iii) Japan.

(b) Upon (i) the date of Sarepta's assumption of Development responsibility for the DM1 Program pursuant to Section 3.1.1(a)(ii) (Arrowhead Category 1 Program Development Responsibility) and Section 3.1.1(b) (Sarepta Category 1 Development Step-In Right) or (ii) the initiation of a [***] pursuant to Section 8.3.1(d) (DM1 First Development Milestone Event) or Section 8.3.1(e) (DM1 Second Development Milestone Event), and (in each case ((i) and (ii)), without limitation of Sarepta's obligations under Section 3.3.2(a) (Sarepta Development Diligence Obligations) for the DM1 Program, Sarepta, either itself or through its Affiliates or Sublicensees, will use Commercially Reasonable Efforts (which, for purposes of this Section 3.3.2(b) (Sarepta Development Diligence Obligations) will not take into consideration payments to Arrowhead under this Agreement) to achieve the DM1 First Development Milestone Event and DM1 Second Development Milestone Event, depending on the stage of the DM1 Program to the extent such milestone events had not been achieved by Arrowhead.

3.4. Arrowhead Development Costs Reimbursement.

3.4.1. In consideration for Arrowhead's performance of the Ongoing C1 Development Activities and, if any, the Additional R&D Activities for any Program in accordance with the applicable Additional R&D Plan, Sarepta will reimburse Arrowhead for the amount of all FTE Costs and, subject to Section 5.1.1 (Arrowhead Manufacturing Activities), all Out-of-Pocket Costs incurred by or on behalf of Arrowhead in the performance of such Ongoing C1 Development Activities and, if any, Additional R&D Activities: (a) from and after the Effective Date for each Program other than the DM1 Program and (b) solely after the date of occurrence of both the DM1 First Development Milestone Event and the DM1 Second Development Milestone Event for the DM1 Program (collectively, the "**Arrowhead Development Costs**"), in each case ((a) and (b)), to the extent such Arrowhead Development Costs do not exceed [***] of the amounts set forth in the corresponding Category 1 Development Budget or Additional R&D Budget (as applicable) set forth in the corresponding Category 1 Development Plan or Additional R&D Plan (as applicable) (the "**Reimbursable Development Costs**"). Within [***] days following the final day of each Calendar Quarter, Arrowhead will issue to Sarepta an invoice for the amount of the Reimbursable Development Costs for each applicable Program incurred by Arrowhead during such Calendar Quarter. Sarepta will reimburse Arrowhead for all undisputed amounts set forth in any such invoice within [***] days after receipt thereof.

3.4.2. In addition to the amounts to be reimbursed by Sarepta for the DM1 Program pursuant to Section 3.4.1 (Arrowhead Development Costs Reimbursement), after the date on which the DM1 Second Development Milestone Event has been achieved, Arrowhead will have the right to issue to Sarepta an invoice for the amount of all FTE Costs and, subject to Section 5.1 (Arrowhead Manufacturing Activities), all Out-of-Pocket Costs incurred by or on behalf of Arrowhead in the performance of Ongoing C1 Development Activities during the period commencing on the Effective Date and continuing until the occurrence of the DM1 Second Development Milestone Event for the DM1 Program. Sarepta will reimburse Arrowhead for all undisputed amounts set forth in any such invoice within [***] days after receipt thereof.

3.5. Sarepta Program Costs.

[***] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED.

3.5.1. **Costs for Category 1 Programs**. For each Category 1 Program, from and after the Effective Date, but without duplication of Section 3.4 (Arrowhead Development Cost Reimbursement), Sarepta will be responsible for 100% of all costs and expenses incurred by or on behalf of Sarepta for the Development of all Licensed C1 Compounds and Licensed C1 Products that are the subject of such Category 1 Program for the Territory.

3.5.2. **Costs for Category 2 Programs**. For each Category 2 Program, from and after the CTA Ready Package Acceptance Date for such Category 2 Program, Sarepta will be responsible for 100% of all costs and expenses incurred by or on behalf of Sarepta for the Development of all Licensed C2 Compounds and Licensed C2 Products that are the subject of such Category 2 Program for the Territory.

3.5.3. **Costs for Category 3 Programs**. For each Category 3 Program, from and after the CTA Ready Package Acceptance Date for such Category 3 Program, Sarepta will be responsible for 100% of all costs and expenses incurred by or on behalf of Sarepta for the Development of all Licensed C3 Compounds and Licensed C3 Products that are the subject of such Category 3 Program for the Territory.

3.6. Licensed Products Research and Development Reports.

3.6.1. **Arrowhead**.

(a) **Category 1 Program Transition**. During the period commencing on the Execution Date and continuing until the date on which the Parties complete all activities under all Transition Plans for the Category 1 Programs, on a [***] basis, Arrowhead will keep Sarepta reasonably informed of the progress of activities with respect to each Category 1 Program by delivering to Sarepta a high-level update of the key Development and Manufacturing activities performed by Arrowhead or its Affiliates or Sublicensees during the preceding week with respect to the Category 1 Programs of the information specified in **Schedule 3.6.1(a)** (Form of Weekly Report), except for any such [***] falling during an Arrowhead company holiday.

(b) **Other Activities**. During the period commencing on the Effective Date and continuing until the dissolution of the JDC, Arrowhead will keep the JDC informed regarding the progress of the Ongoing C1 Development Activities, Additional R&D Activities, Category 2 Program Research Activities, and Category 3 Program Research Activities for all Licensed Compounds and Licensed Products by providing to the JDC reasonably in advance of each meeting of the JDC (or at a different frequency determined by the JDC) a report (i) summarizing results and describing progress made against timelines in all Development Plans or, if any, Additional R&D Plans, and any Ongoing C1 Development Activities, Additional R&D Activities, Category 2 Program Research Activities, and Category 3 Program Research Activities planned to be undertaken for the applicable corresponding Licensed Compounds and Licensed Products prior to the next meeting of the JDC, and (ii) for all Licensed Compounds and Licensed Products, a reasonable summary of results, information, and data generated from Ongoing Development Trials, Ongoing C1 Development Activities, Additional R&D Activities, Category 2 Program Research Activities, and Category 3 Program Research Activities, as are

applicable, for such Licensed Compounds and Licensed Products (each such report, a "**Development Report**"); *provided*, *however*, that if the JDC meets more often than once per month, then Arrowhead will only be required to provide one Development Report to the JDC in such month. In addition, through the Parties' respective Alliance Managers, Arrowhead will promptly share with Sarepta all other material developments and material information that it comes to possess relating to the Research and Development of the Licensed Compounds and Licensed Products conducted by or on behalf of Arrowhead under the applicable Development Plan, including any additional information regarding the Development of the Licensed Products reasonably requested by Sarepta from time to time to the extent and in the form readily available to Arrowhead and able to be disclosed to Sarepta, *provided*, *however*, that Arrowhead will not be required to provide any Arrowhead Excluded Know-How.

3.6.2. **Sarepta**. On a Program-by-Program basis, following the assumption of all Development activities for such Program by Sarepta in accordance with Section 3.2.1 (Sarepta Development and Medical Affairs Responsibility), and until the receipt of Regulatory Approval for the first Licensed Product that is the subject of such Program in the United States or any of the Major European Markets, Sarepta will provide Arrowhead with a reasonably detailed report (which may be in the form of slides) on a semi-annual basis, on or prior to February 15th and August 15th of each Calendar Year, summarizing the material Development activities conducted by Sarepta and its Affiliates and their respective Sublicensees with respect to the Licensed Compounds and the Licensed Products that are the subject of such Program, including (a) material developments with respect to such Licensed Compounds and Licensed Products, including the anticipated timing of achievement of the milestone events set forth in Section 8.3 (Milestone Payments), and (b) any Regulatory Approvals received for such Licensed Products in the Territory. All information in such reports will be deemed Sarepta's Confidential Information.

3.7. Scientific Records. Arrowhead (with respect to the Ongoing C1 Development Activities, any Additional R&D Activities, the Category 2 Program Research Activities, and the Category 3 Program Research Activities for the applicable Programs) and Sarepta (with respect to all other Development activities hereunder, including any Assumed C1 Program Development Activities, for all Programs) will maintain scientific records in sufficient detail and in good scientific manner appropriate for patent and regulatory purposes, and, to the extent applicable, in compliance with GLP, GMP, and GCP with respect to activities intended to be submitted in regulatory filings (including INDs), all of which records will fully and accurately reflect all work done and results achieved in the performance of the Development activities and Clinical Trials by or on behalf of such Party with respect to Licensed Products under this Agreement.

4. REGULATORY MATTERS

4.1. Regulatory Responsibilities.

4.1.1. **Arrowhead Regulatory Responsibilities**.

(a) **Category 1 Programs**. With respect to each Category 1 Program, prior to the CTA Transfer Date for such Category 1 Program, except as otherwise set forth in [***], (i) Arrowhead will be the Regulatory Lead for all regulatory matters in the

Territory relating to such Category 1 Program, will own and maintain all INDs, MAAs, Regulatory Approvals, other Regulatory Submissions, and related regulatory documents in the Territory with respect to the Licensed Compounds and Licensed Products that are the subject of such Category 1 Program (in each case, as applicable), and will be responsible, and act as the point of contact, for communications with all Regulatory Authorities in the Territory relating to Licensed C1 Products that are the subject of such Category 1 Program; and (ii) Arrowhead will be the Regulatory Lead for all meetings with applicable Regulatory Authorities in the Territory related to Licensed C1 Products that are the subject of such Category 1 Program. Subject to and without limitation of Arrowhead's rights under Section 3.1.1(c)(ii) (Material Trial Changes), Arrowhead will provide Sarepta with a copy of all proposed Regulatory Submissions to be filed with or submitted to any Regulatory Authority in connection with the Ongoing Clinical Trials for Sarepta's review and comment sufficiently in advance of Arrowhead's filing or submission thereof, and Arrowhead will incorporate any reasonable comments received from Sarepta into such Regulatory Submissions. To the extent permitted by the applicable Regulatory Authority and applicable Law, Sarepta will be permitted to attend and participate in any meeting between Arrowhead and a Regulatory Authority to the extent related to a Category 1 Program (and any material internal meeting of Arrowhead that is scheduled in advance, in preparation for such meeting with such Regulatory Authority), and Arrowhead will notify Sarepta within [***] after Arrowhead receives notice of such meeting with such Regulatory Authority and will use reasonable efforts to notify Sarepta prior to any such material internal meeting or at least [***] prior to any such meeting with such Regulatory Authority.

(b) **Category 2 Programs and Category 3 Programs**. With respect to each Category 2 Program and Category 3 Program, at least [***] days prior to the anticipated CTA Ready Package Acceptance Date for, respectively, the corresponding Category 2 Program and Category 3 Program, the JDC will meet to discuss the status of such Category 2 Program or Category 3 Program, including the results of all studies conducted in support thereof.

4.1.2. **Sarepta Regulatory Responsibilities**.

(a) [***]

(b) **Category 1 Programs, Category 2 Programs, and Category 3 Programs**. Except as otherwise set forth in [***], with respect to (i) each Licensed C1 Product, from and after the CTA Transfer Date for the applicable Category 1 Program, and (ii) each Licensed C2 Product that is the subject of a given Category 2 Program and each Licensed C3 Product that is the subject of a given Category 3 Program, from and after the CTA Ready Package Acceptance Date for, respectively, the corresponding Category 2 Program and Category 3 Program, in each case ((i) and (ii)), (A) Sarepta (itself or through its Affiliate or Sublicensee) will be the Regulatory Lead for all regulatory matters in the Territory relating to such Licensed Products, will own and maintain all INDs, MAAs, Regulatory Approvals, other Regulatory Submissions, and related regulatory documents, in the Territory with respect to such Licensed Products (in each case, as applicable) and will be

responsible, and act as the point of contact, for communications with all Regulatory Authorities in the Territory relating to such Licensed Product; (B) Sarepta will have sole control over, and decision-making authority with respect to, preparing, filing, and maintaining all INDs, MAAs, Regulatory Approvals, other Regulatory Submissions, and related regulatory documents, for such Licensed Product and, at Sarepta's cost and expense, Arrowhead will provide reasonable support for such INDs, MAAs, Regulatory Approvals, other Regulatory Submissions, and related regulatory documents, as requested by Sarepta; and (C) Sarepta will be the Regulatory Lead for all meetings with all applicable Regulatory Authorities in the Territory related to such Licensed Product.

4.2. Assignment of Regulatory Submissions.

4.2.1. **Clinical Trial Regulatory Submissions**.

(a) **Clinical Trial Regulatory Submissions**. With respect to each Category 1 Program, (i) within [***] days after the date on which Sarepta assumes responsibility and control of the applicable Ongoing Clinical Trial for the Licensed C1 Product that is the subject of such Category 1 Program as an Assumed C1 Program Development Activity or (ii) on such other date as the Parties may agree in writing, Arrowhead will transfer and assign to Sarepta all rights, title, sponsorship, and interests in and to the Clinical Trial Regulatory Submissions for all Licensed C1 Products that are the subject of such Category 1 Program. The date of such transfer, for the applicable Category 1 Program, will be the "**CTA Transfer Date**." Prior to or on the CTA Transfer Date for each Category 1 Program, Arrowhead will, at Sarepta's request, as applicable, submit written notification to the applicable Regulatory Authorities informing them of the transfer from Arrowhead to Sarepta of the Clinical Trial Regulatory Submissions for all applicable Licensed C1 Products that are the subject of such Category 1 Program. Arrowhead will provide to Sarepta any other reasonable support that may be required to effect the change in sponsor for any Ongoing Clinical Trials as of the CTA Transfer Date.

(b) **Other Assigned Regulatory Submissions**. Promptly after the applicable CTA Transfer Date for a given Category 1 Program, Arrowhead will transfer or otherwise make available to Sarepta copies (in electronic or other existing format) of any other Regulatory Submissions in the Territory Controlled by Arrowhead or its Affiliates as of the CTA Transfer Date and not already transferred to Sarepta pursuant to Section 4.2.1(a) (Clinical Trial Regulatory Submissions), to the extent such materials solely relate to Licensed C1 Products that are the subject of such Category 1 Program. Promptly after the completion of all transition activities set forth in this Section 4.2.1(b) (Other Assigned Regulatory Submissions), Arrowhead will send a letter to each applicable Regulatory Authority to record and notify such Regulatory Authority of the transfer to Sarepta of such Regulatory Submissions for such applicable Licensed C1 Products that are the subject of such Category 1 Program.

(c) **Clinical Trial Data**. In connection with the transfer of Regulatory Submissions provided for in this Section 4.2.1 (Clinical Trial Regulatory Submissions),

Arrowhead will provide to Sarepta separate copies (in electronic or other existing format) of the study reports and underlying data (to the extent not previously provided to Sarepta) from the Ongoing C1 Development Activities and Additional R&D Activities.

(d) **Arrowhead Assistance**. Upon Sarepta's reasonable request, on an Ongoing Clinical Trial-by-Ongoing Clinical Trial basis and for a period not to exceed [***] months after the CTA Transfer Date for the Category 1 Program in which Licensed C1 Products are the subject of such Ongoing Clinical Trial, Arrowhead will provide any reasonably necessary support to Sarepta in connection with the corresponding transferred and assigned Clinical Trial Regulatory Submissions to enable Sarepta to prepare and file Regulatory Submissions to, and obtain Regulatory Approvals from, the applicable Regulatory Authorities in the Territory for such Licensed C1 Products, provided that such assistance will be limited to assistance that relates directly to activities that Arrowhead conducted prior to the Effective Date or in connection with such Ongoing Clinical Trial, including all Ongoing C1 Development Activities and Additional R&D Activities. Subject to the terms and conditions of this Agreement, within a reasonable time following Sarepta's request, Arrowhead will execute and deliver, or will cause to be executed and delivered, to Sarepta such endorsements, assignments, and other documents as may be reasonably necessary to assign, convey, transfer, and deliver to Sarepta all of Arrowhead's rights, title, and interests in and to the Assigned Regulatory Submissions. Sarepta will reimburse Arrowhead for (A) any reasonable Out-of-Pocket Costs incurred in providing such assistance and (B) Arrowhead's reasonable costs, at the FTE Rate, incurred in connection with its Personnel's participation in any such assistance activities, including working group meetings or any other one-on-one meetings with Personnel of Sarepta or its designee, in each case ((A) and (B)), solely to the extent such Out-of-Pocket Costs and FTE Costs are set forth in a budget agreed to by the Parties in writing or through the JSC before Arrowhead undertakes the applicable assistance activities.

4.3. Costs of Regulatory Affairs. Sarepta will be solely responsible for all costs and expenses incurred by or on behalf of Sarepta or its Affiliates associated with preparing, filing, obtaining, and maintaining Regulatory Approvals in the Territory for the Licensed Products.

4.4. Pharmacovigilance Agreement. As soon as reasonably practicable following the Effective Date but in no event later than [***] days after the Effective Date, the Parties will use good faith efforts to negotiate and execute a pharmacovigilance agreement, on reasonable and customary terms that may provide for, among other things, (a) the establishment of a joint safety committee that will oversee each Party's activities under such pharmacovigilance agreement as a subcommittee of the JSC and (b) guidelines and responsibilities for (i) the receipt, investigation, recording, review, communication, reporting, and exchange between the Parties of Adverse Event reports and other safety information relating to the Licensed Compounds and Licensed Products, (ii) reconciliation procedures to ensure adequate and compliant exchange of safety data, (iii) contact with Regulatory Authorities with respect to the foregoing, and (iv) the maintenance of a global safety database with respect to the Licensed Compounds and Licensed Products, in each case ((i) – (iv)), in accordance with applicable Law (the "**Pharmacovigilance Agreement**"). The Pharmacovigilance Agreement will contain terms no less stringent than those required by ICH or other applicable guidelines in order to allow the Parties to meet the applicable regulatory and legal requirements regarding the

management of safety data. Pending entry into such Pharmacovigilance Agreement, the Parties will, if necessary, within [***] days following the Effective Date implement an interim procedure for exchange of any and all information concerning all Adverse Events related to use of the Licensed Products. Without limiting the foregoing, Arrowhead will use reasonable efforts to promptly notify Sarepta of any patient safety issue that arise hereunder, and the Parties will promptly meet and discuss such patient safety issue, including any Material Trial Changes with respect thereto.

5. MANUFACTURING

5.1. Arrowhead Manufacturing Activities.

5.1.1. **Category 1 Programs**. For each Category 1 Program and the applicable Ongoing C1 Development Activities and Assumed C1 Program Development Activities, Arrowhead will be responsible for the Manufacture and supply of the clinical requirements of the applicable Category 1 Lead Compound that is the subject of such activities for the conduct of such activities until the later of (a) the date of Arrowhead's completion of the Ongoing C1 Development Activities and, if applicable, of any Additional R&D Activities for such Category 1 Program, (b) the date of Sarepta's completion of the Assumed C1 Program Development Activities for such Category 1 Program if Sarepta has exercised its right to assume such activities as set forth in Section 3.1.1(a) (Arrowhead Category 1 Development Responsibility), and (c) [***]. The costs of such Manufacturing activities conducted by Arrowhead for such Category 1 Program will be borne by Arrowhead for so long as Arrowhead is sourcing such supply from inventory of the applicable Category 1 Lead Compound that was Manufactured prior to the Effective Date, and, after such supply has been exhausted, will be reimbursed by Sarepta as Arrowhead Development Costs in accordance with Section 3.4.1 (Arrowhead Development Costs Reimbursement) to the extent that such costs are not included in the Fully Burdened Costs and reimbursed by Sarepta pursuant to clause (a) of Section 5.2 (Arrowhead Supply Obligations).

5.1.2. **Category 2 Programs**. For each Category 2 Program and its Category 2 Program Research Activities, Arrowhead will be responsible for the Manufacture and supply of the clinical requirements of the applicable Licensed C2 Compounds that are the subject of such activities for the conduct of such activities until the CTA Ready Package Acceptance Date for such Category 2 Program. The costs of such Manufacturing activities conducted by Arrowhead will be borne by Arrowhead.

5.1.3. **Category 3 Programs**. For each Category 3 Program and its Category 3 Program Research Activities, Arrowhead will be responsible for the Manufacture and supply of the clinical requirements of the applicable Licensed C3 Compounds that are the subject of such activities for the conduct of such activities until the CTA Ready Package Acceptance Date for such Category 3 Program. The costs of such Manufacturing activities conducted by Arrowhead will be borne by Arrowhead.

5.2. Arrowhead Supply Obligation. Without limiting Sarepta's right to request and have completed a Manufacturing technology transfer pursuant to Section 5.4 (Manufacturing Technology Transfer), (a) after the completion of the Manufacturing activities described in Section 5.1 (Arrowhead Manufacturing Activities) for a Program, Arrowhead will Manufacture and supply to Sarepta and its Affiliates and Sublicensees all clinical requirements necessary for Development of any of the

Licensed Compounds and Licensed Products that are the subject of such Program in the Territory at [***], and (b) (i) with respect to the Category 1 Programs, Arrowhead will Manufacture and supply to Sarepta and its Affiliates and Sublicensees all commercial requirements necessary for Commercialization of any of the Licensed C1 Products in the Territory at [***]; and (ii) with respect to the Category 2 Programs and Category 3 Programs, Sarepta will discuss with Arrowhead having Arrowhead Manufacture and supply to Sarepta and its Affiliates and Sublicensees any or all commercial requirements necessary for Commercialization of any of the Licensed C2 Products or Licensed C3 Products in the Territory at [***], in each case ((a) and (b)), of (A) the active pharmaceutical ingredient or drug substance for such Licensed Product ("**Drug Substance**") and (B) if further requested by Sarepta, the drug product form of such Licensed Product ("**Drug Product**"). Within (1) with respect to clinical supply for the Category 1 Programs and Category 2 Programs, [***] days after the Effective Date, (2) with respect to commercial supply for a Category 1 Program, a reasonable time in advance of the anticipated date of receipt of the first Regulatory Approval for the first Licensed C1 Product that is the subject of such Category 1 Program, or (3) with respect to commercial supply for a Category 2 Program or Category 3 Program or clinical supply for a Category 3 Program, a reasonable time after the Parties agree that Arrowhead will Manufacture and supply for such Category 2 Program or Category 3 Program, in each case ((1)-(3)), the Parties will negotiate in good faith and, upon agreement thereof, execute a clinical supply agreement or a commercial supply agreement, as applicable, on reasonable and customary terms for such agreements, and a related quality agreement. If the Parties, acting in good faith, are unable to agree on any such supply agreement or quality agreement within [***] days following the date of Sarepta's written notice (as may be extended by agreement of the Parties), then either Party may refer such matter dispute to be resolved in accordance with Section 15.3 (Expedited Arbitration). As will be agreed by the Parties upon Sarepta's request, and further described in the applicable supply agreement, Arrowhead will [***]. In addition, in the applicable supply agreements, Arrowhead will agree that [***]. For clarity, whether Arrowhead engages in clinical or commercial Manufacture and supply pursuant to this Section 5.2 (Arrowhead Supply Obligation), Sarepta will be solely responsible, itself or through an Affiliate or a CMO, for final packaging and labelling of the Drug Product for each of the Licensed Products for the Territory.

5.3. Remaining Inventory.

5.3.1. **Licensed C1 Compounds**. For each Category 1 Program, upon the earlier of (a) the later of the date of Arrowhead's completion of the Ongoing C1 Development Activities and, if applicable, of any Additional R&D Activities for such Category 1 Program, and (b) the date of Sarepta's assumption of the Assumed C1 Program Development Activities for such Category 1 Program, Arrowhead will promptly deliver to Sarepta, upon the written request of Sarepta, any and all remaining inventory of the Licensed C1 Compound of such Category 1 Program that is in Arrowhead's possession as of such date: [***].

5.3.2. **Licensed C2 Compounds and Licensed C3 Compounds**. For each Category 2 Program and Category 3 Program, upon the CTA Ready Package Acceptance Date for, respectively, the corresponding Category 2 Program and Category 3 Program, Arrowhead will promptly deliver to Sarepta, upon the written request of Sarepta, any and all remaining inventory of the Licensed C2 Compounds or Licensed C3 Compounds of, respectively, the corresponding Category 2 Program and Category 3 Program that is in Arrowhead's possession as of such date: [***].

[***] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED.

5.4. Manufacturing Technology Transfer. With respect to the Category 2 Programs and Category 3 Programs, at any time after the Effective Date, and, with respect to the Category 1 Programs, only if Arrowhead is unable or unwilling to Manufacture for Sarepta any Licensed C1 Compound or Licensed C1 Product, or Arrowhead is in material breach of, or there is a supply failure (as defined therein) under, the relevant clinical or commercial supply agreement with respect to such Licensed C1 Compound or Licensed C1 Product, Sarepta may request in writing the transfer from Arrowhead or Arrowhead's CMO to Sarepta or (a) any CMO set forth on **Schedule 5.4** (Approved Sarepta CMO) or (b) with Arrowhead's prior written consent (not to be unreasonably withheld, conditioned or delayed) any other CMO designated by Sarepta (the CMOs set forth on **Schedule 5.4** (Approved Sarepta CMO) and any such other Arrowhead approved CMOs, each, an "**Approved Sarepta CMO**"), copies or samples of all Arrowhead Know-How that is necessary or reasonably useful to enable the Manufacture of the Licensed Compounds and Licensed Products (the "**Arrowhead Manufacturing Know-How**"). Promptly following any such written request by Sarepta and within [***] days after such written request, the JMC will prepare, and submit to the JSC to review, discuss, and determine whether to approve, a written Manufacturing technology transfer plan that provides for (i) Arrowhead or Arrowhead's Third Party CMO transferring copies of relevant documentation, samples of Drug Substance, and copies of other embodiments of such Arrowhead Manufacturing Know-How (including data within reports, notebooks, and electronic files), and (ii) Arrowhead making available its technical Personnel on a reasonable basis and as more specifically specified therein to consult with Sarepta with respect to such transferred Arrowhead Manufacturing Know-How (such plan, the "**Manufacturing Technology Transfer Plan**"). The Manufacturing Technology Transfer Plan will include a budget of the Out-of-Pocket Costs (including any amounts that Arrowhead will pay to its Subcontractors, including CMOs) to be incurred by Arrowhead in the performance of the activities set forth in the Manufacturing Technology Transfer Plan (the "**Manufacturing Technology Transfer Budget**"). Pursuant to the timelines set forth in the Manufacturing Technology Transfer Plan that is agreed to by the Parties, and in any event no later than [***] days thereafter, Arrowhead will work with Sarepta to complete the transfer of the Arrowhead Manufacturing Know-How and other activities set forth in the Manufacturing Technology Transfer Plan on the timelines and in accordance with the budget set forth therein. Sarepta will reimburse Arrowhead for its Out-of-Pocket Costs, but for clarity not its FTE Costs, incurred in completing such transfer in accordance with the Manufacturing Technology Transfer Plan in accordance with the Manufacturing Technology Transfer Budget. Any additional assistance to be provided by Arrowhead in connection with Arrowhead Know-How that has been transferred pursuant to a completed Manufacturing Technology Transfer Plan may be requested by Sarepta and provided by Arrowhead pursuant to a separate written agreement between the Parties providing for reasonable compensation to be paid to Arrowhead for providing such additional assistance.

6. COMMERCIALIZATION

6.1. Commercialization of the Licensed Products.

6.1.1. **Commercialization Diligence Obligations**. On a Program-by-Program basis, following receipt by Sarepta or its Affiliates or Sublicensees of Marketing Approval for a Licensed Product that is the subject of a given Program in the applicable country, Sarepta, either itself or through its Affiliates or Sublicensees, will use Commercially Reasonable Efforts to Commercialize at least one Licensed Product that is the subject of such Program in each of: (a) the United States, (b) any three Major European Markets, and (c) Japan.

6.1.2. **Commercialization Responsibility**. As between the Parties, Sarepta will lead and have sole control over and decision-making authority with respect to all Commercialization activities for the Licensed Products in the Territory, at its sole cost and expense.

6.2. Reporting Obligations. Sarepta will report to Arrowhead in writing, on an annual basis in the first Calendar Quarter of each Calendar Year, beginning with the Calendar Year following receipt of the first Regulatory Approval of each Licensed Product (for the period ending December 31 of the prior Calendar Year), summarizing in reasonable detail Sarepta's and its Affiliates' and its and their Sublicensees' Commercialization activities for such Licensed Product performed to date (or updating such report for activities performed since the last such report was given hereunder, as applicable). In addition, Sarepta will provide Arrowhead with written notice of the First Commercial Sale of each Licensed Product in the Territory as soon as reasonably practicable after such event; *provided* that Sarepta will inform Arrowhead of such event prior to public disclosure of such event by Sarepta or its Affiliates or its and their Sublicensees. All information in such reports will be deemed Sarepta's Confidential Information.

6.3. Recalls, Market Withdrawals, or Corrective Actions. Each Party will use reasonable efforts to notify the other Party promptly, but in no event later than five Business Days, following its determination that any event, incident, or circumstance has occurred that may result in the need for a recall, market suspension, or market withdrawal of a Licensed Product in the Territory and will include in such notice the reasoning behind such determination. Sarepta will have the sole right to make the final determination as to whether to voluntarily implement any such recall, market suspension, or market withdrawal in the Territory; *provided* that prior to the implementation of such a recall, market suspension, or market withdrawal, to the extent practical, Sarepta will consult with Arrowhead and will consider Arrowhead's comments in good faith. Except as otherwise set forth in the applicable supply agreement and its corresponding quality agreement, for all recalls, market suspensions, or market withdrawals undertaken pursuant to this Section 6.3 (Recalls, Market Withdrawals, or Corrective Actions) after the applicable CTA Transfer Date for the applicable Licensed Product, Sarepta will be solely responsible for the execution thereof, and, at Sarepta's cost and expense, Arrowhead will reasonably cooperate in all such efforts, unless, such recall, market suspension, or market withdrawal was the result of Arrowhead's failure to supply the applicable Licensed Product to Sarepta in accordance with the specifications set forth for such product under the applicable supply agreement (including manufacture of such Licensed Product in accordance with GMP), in which case, Arrowhead will be responsible for all costs and expenses incurred to conduct such recall, market suspension, or market withdrawal (including provision reasonable cooperation to Sarepta in connection therewith).

7. GOVERNANCE

7.1. Alliance Managers. Promptly following the Effective Date, each Party will designate an individual to facilitate communication and coordination of the Parties' activities under this Agreement relating to the Development and Manufacture of Licensed Compounds and Licensed Products (each, an "**Alliance Manager**"). For clarity, an Alliance Manager will not be a representative of its respective Party on any committee, and will have no voting right on any committee, unless otherwise agreed in writing by the Parties.

7.2. Joint Steering Committee.

7.2.1. **Formation; Composition; Dissolution**. Within [***] days after the Effective Date, the Parties will establish a committee (the "**Joint Steering Committee**" or "**JSC**") to provide strategic oversight of the Parties' activities under this Agreement. Each Party will initially appoint [***] representatives to the JSC, with each representative having knowledge and expertise in the Development and Manufacture of compounds and products similar to the Licensed Compounds and Licensed Products and having sufficient decision-making authority and seniority within the applicable Party to provide meaningful input and make decisions arising within the scope of the JSC's responsibility. The JSC may change its size from time to time by agreement of the Parties, *provided* that the JSC will consist at all times of an equal number of representatives of each of Arrowhead and Sarepta. Each Party may replace its JSC representatives at any time upon written notice to the other Party. The JSC will be chaired by a chairperson designated by Sarepta. The JSC chairperson may invite non-members to participate in the discussions and meetings of the JSC, if necessary, provided that such participants have no voting authority at the meetings of the JSC and are bound under enforceable obligations of confidentiality and non-use no less protective of the Parties' Confidential Information than those set forth in this Agreement. The JSC chairperson's responsibilities will include conducting meetings, including, when feasible, ensuring that objectives for each meeting are set and achieved. The JSC will dissolve upon the dissolution of the JDC, the JMC, and any other subcommittees.

7.2.2. **Specific Responsibilities of the JSC**. The JSC will have the following responsibilities:

(a) reviewing, discussing, and determining whether to approve the Technology Transfer Plan as set forth in Section 2.4 (Initial Technology Transfer);

(b) discussing and determining whether Sarepta may assume responsibility and control of the Ongoing Development Trials and associated Ongoing C1 Development Activities as set forth in Section 3.1.1(a) (Arrowhead Category 1 Program Development Responsibility);

(c) reviewing, discussing, and determining whether to approve a Category 1 Development Plan and any updates thereto as set forth in Section 3.1.1(c)(i) (Category 1 Development Plans);

(d) reviewing, discussing, and determining whether to implement any Material Trial Change [***], other than (i) an Urgent Material Trial Change as set forth in Section 3.1.1(c)(ii) (Material Trial Changes) or (ii) a Material Trial Change described in clause (B) of Section 3.1.1(c)(ii) (Material Trial Changes);

(e) reviewing, discussing, and determining whether to approve a Category 2 Development Plan and any updates thereto as set forth in Section 3.1.2(b) (Category 2 Development Plans);

(f) reviewing, discussing, and determining whether to approve a Category 3 Development Plan and any updates thereto as set forth in Section 3.1.3(d) (Category 3 Development Plans);

(g) resolving any dispute between the Parties as to whether a Category 2 CTA Ready Package is missing Category 2 CTA Ready Data as set forth in Section 3.1.2(c) (Sarepta Category 2 CTA Ready Package Acceptance);

(h) resolving any dispute between the Parties as to the achievement of the [***] with respect to any Category 3 Program as set forth in Section 3.1.3(c) (Arrowhead Category 3 Development Responsibility);

(i) resolving any dispute between the Parties as to whether [***] as set forth in Section 8.3.1(g) ([***]);

(j) resolving any dispute between the Parties as to whether a Category 3 CTA Ready Package is missing Category 3 CTA Ready Data as set forth in Section 3.1.3(e) (Sarepta Category 3 CTA Ready Package Acceptance);

(k) reviewing, discussing, and determining whether to approve an Additional R&D Plan and any updates thereto as set forth in Section 3.1.4 (Additional R&D Responsibilities);

(l) reviewing, discussing, and determining whether to approve a Transition Plan and any updates thereto as set forth in Section 3.2.2(a)(i)(Transition Plans) and Section 3.2.2(a)(ii) (Transition Plans);

(m) reviewing, discussing, and determining whether to approve a budget of costs and expenses to be incurred by Arrowhead in connection with (i) transition assistance as set forth in Section 3.2.2(b) (Transition Assistance) or (ii) assistance with Sarepta's preparation and filing of Clinical Trial Regulatory Submissions as set forth in Section 4.2.1(d) (Arrowhead Assistance);

(n) reviewing, discussing, and determining whether to approve the Manufacturing Technology Transfer Plan or any updates thereto as set forth in Section 5.4 (Manufacturing Technology Transfer);

(o) reviewing, discussing, and determining whether to approve a Sarepta Research Plan and any updates thereto as set forth in Section 3.2.4 (Sarepta Research Activities);

(p) discussing and determining whether to approve [***];

(q) reviewing, discussing, and determining whether to approve [***];

(r) reviewing, discussing, and determining whether to approve a [***];

(s) reviewing, discussing, and determining how to resolve any issue escalated by, or disputes within, a JDC, the JCC, or the JMC;

(t) establishing such additional committees or subcommittees of the JSC as it deems necessary to oversee activities relating to the Licensed Compounds and Licensed Products under this Agreement, including the joint safety committee as described in Section 4.4 (Pharmacovigilance Agreement); and

(u) performing such other functions expressly allocated to the JSC in this Agreement or by the written agreement of the Parties.

7.2.3. **Meetings**. The JSC will meet at least once every Calendar Quarter, unless the Parties agree in writing to a different frequency. The JSC may meet in person, by videoconference, or by teleconference, but at least one meeting of the JSC per Calendar Year will be in person unless the Parties otherwise agree in writing. In-person JSC meetings will be held at locations alternately selected by Arrowhead and by Sarepta, or at any other location agreed by the members of the JSC. The first JSC meeting will be held within [***] Business Days following the Effective Date. Meetings of the JSC will be effective only if a quorum is present, which quorum will require the presence of at least one representative from each Party. Each Party will bear the expense of its respective JSC members' participation in JSC meetings. No later than [***] Business Days prior to any meeting of the JSC (or such shorter time period as the Parties may agree), the JSC chairperson will work with the Alliance Managers to prepare and circulate an agenda for such meeting. Additional topics may be included on such agenda prior to the meeting, and the Party or the committee proposing an item will provide materials to the JSC representatives no later than five Business Days prior to the JSC meeting to support discussion. The JSC chairperson may also call a special meeting of the JSC (by videoconference, teleconference, or in person) if the JSC chairperson reasonably believes that a significant matter must be addressed prior to the next scheduled meeting, in which event such JSC chairperson will work with the Alliance Managers to provide the members of the JSC, promptly after the decision is made to hold such special JSC meeting, with an agenda for the meeting and materials reasonably adequate to enable an informed decision. The Alliance Managers working with the JSC chairperson will be responsible for preparing reasonably detailed written minutes of JSC meetings that reflect all decisions made and action items identified at such meetings within [***] Business Days after each JSC meeting, and endeavor to finalize such minutes within 30 days after each JSC meeting.

7.2.4. **Decision-Making**. The JSC endeavor to reach decisions by consensus, with each Party, through its representative members of the JSC, having one vote. Approvals of the JSC will require the unanimous agreement of the representatives. If the JSC cannot reach unanimous agreement on an issue that comes before the JSC within [***] Business Days after the meeting at which such issue was raised and over which the JSC has oversight, then the Parties will refer such issue for resolution in accordance with Section 7.5 (Resolution of Committee Disputes).

7.3. Joint Development Committees.

7.3.1. **Formation; Composition; Dissolution**. Within [***] days after the Effective Date, the Parties will establish (a) a committee to coordinate the research and pre-clinical Development of the Licensed Compounds and Licensed Products in the Territory, and (b) a committee to coordinate the clinical Development of the Licensed Compounds and Licensed Products in the Territory (each, a "**Joint Development Committee**" or "**JDC**"). Each Party will initially appoint [***] representatives to each JDC, with each representative having knowledge and expertise in the Development of compounds and products similar to, as applicable, the applicable Licensed Compounds and Licensed Products, and having sufficient seniority and decision-making authority within the applicable Party to provide meaningful input and make decisions arising within the scope

of such JDC's responsibilities. Each Party's JDC representatives may serve on one or more JDCs. Each JDC may change its size from time to time by agreement of the Parties, *provided* that each JDC will consist at all times of an equal number of representatives of each of Arrowhead and Sarepta. Each Party may replace its JDC representatives at any time upon written notice to the other Party. Each JDC may invite non-members to participate in the discussions and meetings of such JDC, *provided* that such participants have no voting authority at the meetings of such JDC and are bound under enforceable obligations of confidentiality and non-use no less protective of the Parties' Confidential Information than those set forth in this Agreement. Each JDC will be chaired by a chairperson designated by Sarepta whose responsibilities will include conducting meetings, including, when feasible, ensuring that objectives for each meeting are set and achieved. The respective applicable JDC will exist for so long as at least one Licensed Compound or Licensed Product is being Developed under this Agreement.

7.3.2. **Specific Responsibilities of the JDCs**. The respective applicable JDC will have the following responsibilities:

(a) preparing the Technology Transfer Plan for discussion, review, and approval by the JSC as set forth in Section 2.4 (Initial Technology Transfer);

(b) preparing a Category 1 Development Plan and any updates thereto, in each case, for discussion, review, and approval by the JSC as set forth in Section 3.1.1(c)(i) (Category 1 Development Plans);

(c) reviewing and discussing all material information relevant to a proposed Material Trial Change as set forth in Section 3.1.1(c)(ii) (Material Trial Changes);

(d) preparing a Category 2 Development Plan and any updates thereto, in each case, for discussion, review, and approval by the JSC as set forth in Section 3.1.2(b) (Category 2 Development Plans);

(e) preparing a Category 3 Development Plan and any updates thereto, in each case, for discussion, review, and approval by the JSC as set forth in Section 3.1.3(d) (Category 3 Development Plans);

(f) preparing an Additional R&D Plan and any updates thereto, in each case, for discussion, review, and approval by the JSC as set forth in Section 3.1.4 (Additional R&D Responsibilities);

(g) preparing a Transition Plan and any updates thereto, in each case, for discussion, review, and approval by the JSC as set forth in Section 3.2.2(a)(i) (Transition Plans) and Section 3.2.2(a)(ii) (Transition Plans);

(h) overseeing the transition of Development activities for each Category 1 Program in accordance with its applicable Transition Plan as set forth in Section 3.2.2(a)(iii) (Transition Plans);

(i) preparing [***];

(j) determining whether to reduce the frequency of Arrowhead's reports summarizing Arrowhead's Development activities under this Agreement as set forth in Section 3.6.1(b) (Arrowhead);

(k) preparing [***];

(l) preparing a [***]; and

(m) performing such other functions expressly allocated to the JDC in this Agreement or by the written agreement of the Parties.

7.3.3. **Meetings**. Each JDC will meet at least once every [***], unless the Parties agree in writing to a different frequency. Each JDC may meet in person, by videoconference, or by teleconference, but that at least one meeting of each JDC per Calendar Year will be in person unless the Parties otherwise agree in writing. In-person JDC meetings will be held at locations alternately selected by Arrowhead and by Sarepta, or at any other location agreed by the members of the respective applicable JDC. Meetings of each JDC will be effective only if a quorum is present, which quorum will require the presence of at least one representative of each Party. Each Party will bear the expense of its respective JDC members' participation in JDC meetings. No later than [***] days prior to the first meeting of the respective applicable JDC in the 2024 stub-Calendar Year and in each Calendar Year thereafter while such JDC exists, the chairperson for such JDC will prepare a communication plan setting forth a schedule of the dates of each meeting of such JDC for that Calendar Year (a "**JDC Communication Plan**"). No later than [***] Business Days prior to any meeting of the respective applicable JDC (or such shorter time period as the Parties may agree), the chairperson of such JDC will work with the Alliance Managers to prepare and circulate an agenda for such meeting. Additional topics may be included on such agenda prior to such meeting, and the Party proposing an item will provide detailed materials to the representatives of such JDC no later than [***] Business Days prior to the JDC meeting to support discussion. A JDC chairperson may also call a special meeting of its JDC (by videoconference, teleconference, or in person) if such JDC chairperson reasonably believes that a significant matter must be addressed prior to the next scheduled meeting, in which event such JDC chairperson will work with the Alliance Managers to provide the members of such JDC, promptly after the decision is made to hold such special JDC meeting, with an agenda for the meeting and materials reasonably adequate to enable an informed decision. The Alliance Managers and the chairperson of the applicable JDC will be responsible for preparing reasonably detailed written minutes of meetings of such JDC that reflect all decisions made and action items identified at such meetings within [***] Business Days after each meeting of such JDC, and endeavor to finalize such minutes within 30 days after each meeting of such JDC.

7.3.4. **Decision-Making**. The JDCs will endeavor to reach decisions by consensus, with each Party, through its representative members of the JSC, having one vote. Approvals of each respective applicable JDC matter will require the unanimous agreement of the representatives of the applicable JDC. If a JDC cannot reach unanimous agreement on a matter issue that comes before it within 10 Business Days following the meeting at which such issue was raised and over which such JDC has oversight, then the Parties will refer such issue for resolution to the JSC pursuant to Section 7.5.1 (Referral to the JSC).

7.4. Joint Manufacturing Committee.

7.4.1. **Formation; Composition; Dissolution**. Within [***] Business Days after the Effective Date, the Parties will establish a committee to coordinate and oversee Manufacturing activities in connection with the Development of the Licensed Compounds and Licensed Products for the Territory (each, a "**Joint Manufacturing Committee**" or "**JMC**"). Each Party will initially appoint [***] representatives to the JMC, with each representative having knowledge and expertise in the performance of Manufacturing activities with respect to compounds and products similar to the applicable Licensed Compounds and Licensed Products, and having sufficient seniority and decision-making authority within the applicable Party to provide meaningful input and make decisions arising within the scope of such JMC's responsibilities. The JMC may change its size from time to time by agreement of the Parties, *provided* that the JMC will consist at all times of an equal number of representatives of each of Arrowhead and Sarepta. Each Party may replace its JMC representatives at any time upon written notice to the other Party. The JMC may invite non-members to participate in the discussions and meetings of the JMC, *provided* that such participants have no voting authority at the meetings of the JMC and are bound under enforceable obligations of confidentiality and non-use no less protective of the Parties' Confidential Information than those set forth in this Agreement. The JMC will be chaired by co-chairpersons designated by Arrowhead and Sarepta, respectively, whose responsibilities will include conducting meetings, including, when feasible, ensuring that objectives for each meeting are set and achieved. The JMC will exist for so long as there are Manufacturing activities being conducted or planned to be conducted for at least one Licensed Compound or Licensed Product under this Agreement.

7.4.2. **Specific Responsibilities of the JMC**. Subject to any limitations under applicable Law, the JMC will have the following responsibilities:

(a) discuss Manufacturing activities to be performed by or on behalf of Arrowhead under any applicable clinical supply agreement or commercial supply agreement;

(b) preparing the Manufacturing Technology Transfer Plan and any updates thereto, in each case, for discussion, review, and approval by the JSC as set forth in Section 5.4 (Manufacturing Technology Transfer);

(c) overseeing the transfer of the Arrowhead Manufacturing Know-How and other activities set forth in the Manufacturing Technology Transfer Plan as set forth in Section 5.4 (Manufacturing Technology Transfer); and

(d) performing such other functions expressly allocated to the JMC in this Agreement or by the written agreement of the Parties.

7.4.3. **Meetings**. The JMC will meet at least once every [***], unless the Parties agree in writing to a different frequency, and otherwise as agreed by the Parties with respect to Manufacturing activities-specific matters. The JMC may meet in person, by videoconference, or by teleconference, but at least one meeting of the JMC per Calendar Year will be in person unless the Parties otherwise agree in writing. In-person JMC meetings will be held at locations alternately selected by Arrowhead and by Sarepta, or at any other location agreed by the members of the JMC. Meetings of the JMC will be

effective only if a quorum is present, which quorum will require the presence of at least one representative of each Party. Each Party will bear the expense of its respective JMC members' participation in JMC meetings. No later than [***] days prior to the first meeting of the JMC in the 2024 stub-Calendar Year and in each Calendar Year thereafter while the JMC exists, the co-chairpersons for the JMC will prepare a communication plan setting forth a schedule of the dates of each meeting for the JMC for that Calendar Year (a "**JMC Communication Plan**"). No later than [***] Business Days prior to any meeting of the JMC (or such shorter time period as the Parties may agree), the co-chairpersons of the JMC will work with the Alliance Managers to prepare and circulate an agenda for such meeting. Additional topics may be included on such agenda, prior to the meeting, and the Party proposing an item will provide materials to the representatives of the JMC no later than [***] Business Days prior to the JMC meeting to support discussion. A JMC co-chairperson may also call a special meeting of the JMC (by videoconference, teleconference, or in person) if such JMC co-chairperson reasonably believes that a significant matter must be addressed prior to the next scheduled meeting, in which event such JMC co-chairperson will work with the Alliance Managers to provide the members of the JMC, promptly after the decision is made to hold such special JMC meeting, with an agenda for the meeting and materials reasonably adequate to enable an informed decision. The Alliance Managers and the co-chairperson of the JMC will be responsible for preparing reasonably detailed written minutes of meetings of the JMC that reflect all decisions made and action items identified at such meetings within [***] Business Days after such meeting of the JMC, and endeavor to finalize such minutes within [***] days after each meeting of the JMC.

7.4.4. **Decision-Making**. The JMC will endeavor to reach decisions by consensus, with each Party, through its representative members of the JMC, having one vote. Approvals of each respective applicable JMC matter will require the unanimous agreement of the representatives of the JMC. If the JMC cannot reach unanimous agreement on a matter that comes before it within [***] Business Days following the meeting at which such issue was raised and over which such JMC has oversight, then the Parties will refer such issue for resolution to the JSC pursuant to Section 7.5.1 (Referral to the JSC).

7.5. Resolution of Committee Disputes.

7.5.1. **Referral to the JSC**. If any subcommittee or working group of a JDC or the JMC or any additional committees or subcommittees formed by the JSC cannot reach consensus on any matter within its decision-making authority within [***] Business Days after the meeting at which such failure to reach consensus occurred, then such matter will first be referred for attempted resolution to the applicable committee. If a JDC, the JMC, or any other committee or subcommittee of the JSC cannot reach consensus on any matter within its decision-making authority within [***] Business Days after the meeting at which such failure to reach consensus occurred, then the matter will be referred for attempted resolution to the JSC.

7.5.2. **Referral to Executive Officers**. If the JSC cannot reach a consensus decision under Section 7.5.1 (Referral to the JSC), then the matter will be referred to the Executive Officers within [***] Business Days after its determination under Section 7.5.1 (Referral to the JSC) that a consensus cannot be reached. If a matter is referred to the Executive Officers under this Section 7.5.2 (Referral to Executive Officers), then the JSC will submit

in writing to their respective Executive Officers the respective positions of the Parties. Such Executive Officers will use good faith efforts to resolve such matter promptly, which good faith efforts will include at least one meeting between such Executive Officers within five Business Days after such chairperson's submission of their respective positions on such matter to them.

7.5.3. **Final Decision-Making Authority**. If the Executive Officers are unable to reach unanimous agreement on any such matter within [***] Business Days after the meeting between the Executive Officers, then, subject to Section 7.5.4 (Exercise of Decision-Making Authority), the following will apply:

(a) if the escalated matter relates to a dispute over (i) whether the [***] have been met with respect to a Category 3 Program, or (ii) [***], then, in each case ((i) and (ii)):

(i) each Party will appoint an independent Third Party expert having at least 15 years of pharmaceutical and biotechnology industry experience and such Third Party experts will appoint a third independent Third Party expert (the "**Third Party Experts**") to resolve such matter;

(ii) each Party will be entitled, within [***] Business Days after the appointment of the final Third Party Expert, to make a written submission to the Third Party Experts explaining the basis for such Party's position;

(iii) the Third Party Experts will render a decision on such matter within [***] Business Days after such Third Party Experts' receipt of the last such written submission by the Parties, which decision will be final and binding on the Parties; and

(iv) each Party will be responsible for its own costs and expenses; *provided*, *however*, that the fees of the Third Party Experts will be borne by the Party against which the Third Party Experts decide;

(b) if the escalated matter relates to approval of (i) [***], (ii) [***], (iii) [***], or (iv) [***], then neither Party will have final decision-making authority with respect to such matter [***];

(c) if the escalated matter relates to [***], then [***] neither Party will have final decision-making authority with respect to such matter and the Parties will continue operating under the *status quo* prior to such dispute [***];

(d) if the escalated matter relates to [***], then Arrowhead will have final decision-making authority with respect to such matter; and

(e) if the escalated matter relates to [***], then [***] Sarepta will have final decision-making authority with respect to such matter.

7.5.4. **Exercise of Decision-Making Authority**. No exercise of a Party's decision-making authority on any matters may, without the other Party's prior written consent, (a) unilaterally waive its own compliance with, modify, or amend the terms or conditions of this Agreement; (b) otherwise conflict with this Agreement; (c) approve any amendment

to a Development Plan, Additional R&D Plan, Transition Plan, the Manufacturing Technology Transfer Plan, or the Technology Transfer Plan, in each case, that would [***], (d) approve any initial Development Plan for a Category 1 Program or Category 2 Program that would [***]; (e) approve any amendment to a Development Plan, Additional R&D Plan, Transition Plan, the Manufacturing Technology Transfer Plan, or the Technology Transfer Plan, in each case, that would [***]; or (f) result in a material change of the day-to-day use or operational allocation of such Person's personnel, equipment, and resources.

7.6. General Committee Authority. Each committee has solely the powers expressly assigned to it in this Article 7 (Governance). No committee will have any power to amend, modify, or waive the terms or conditions of this Agreement or compliance with the terms and conditions of this Agreement.

8. PAYMENTS

8.1. Upfront Payment. In consideration of the licenses and other rights granted to Sarepta hereunder, within [***] days following the Effective Date, Sarepta will make a non-refundable and non-creditable upfront payment to Arrowhead of $500,000,000 via wire transfer of immediately available funds to a U.S. bank account that has been designated by Arrowhead prior to the Effective Date (the "**Upfront Payment**").

8.2. Annual Fees. In consideration of the licenses and other rights granted to Sarepta hereunder, during the Term, upon each of the first five anniversaries of the Effective Date, Sarepta will make a non-refundable and non-creditable payment to Arrowhead of $50,000,000 via wire transfer of immediately available funds to a U.S. bank account that has been designated by Arrowhead prior to the date of such payment (the "**Annual Fees**"); *provided*, *however*, that no Annual Fee will be payable by Sarepta from and after the date on which (a) Sarepta has delivered to Arrowhead a written notice of termination pursuant to Section 13.5.1 (Material Breach or Cure Period); *provided* that if this Agreement is not actually terminated by Sarepta following delivery of such notice of termination for material breach (whether because Arrowhead cures such material breach during the applicable Cure Period or it is determined that Arrowhead was not in material breach of this Agreement), then Sarepta will pay to Arrowhead any Annual Fees that would have otherwise become due following delivery of such notice of termination for material breach *plus* interest thereon in accordance with Section 8.7.7 (Interest Due); or (b) Sarepta has delivered to Arrowhead a written notice of termination pursuant to Section 13.3 (Termination for Convenience). For clarity, the maximum amount payable by Sarepta to Arrowhead under this Section 8.2 (Annual Fees) is $250,000,000.

8.3. Milestone Payments.

8.3.1. **DM1 Program Development Milestones**.

(a) **DM1 Program Development Milestones**. With respect to the DM1 Program, Sarepta will make one-time, non-refundable [***], and non-creditable milestone payments to Arrowhead of: (i) $100,000,000 upon the achievement of the DM1 First Development Milestone Event (the "**DM1 First Development Milestone Payment**"), and (ii) $200,000,000 upon the achievement of the DM1 Second Development Milestone Event (the "**DM1 Second Development Milestone**

Payment") in accordance with Section 8.3.1(b) (DM1 Program Development Milestones).

(b) **Manner of Payment**. Arrowhead will notify Sarepta in writing, or, if Sarepta has assumed Development responsibility for the DM1 Program pursuant to Section 3.1.1(a)(ii) (Arrowhead Category 1 Program Development Responsibility) or Section 3.1.1(b) (Sarepta Category 1 Development Step-In Right), then, in each case, Sarepta will notify Arrowhead in writing, of the achievement of the DM1 First Development Milestone Event as set forth in Section 8.3.1(d) (DM1 First Development Milestone Event) and the DM1 Second Development Milestone Event as set forth in Section 8.3.1(e) (DM1 Second Development Milestone Event), in each case, no later than [***] days after such Party's achievement thereof. Sarepta will pay the milestone payment owed to Arrowhead pursuant to, as applicable, Section 8.3.1(a)(i) (DM1 Program Development Milestones) or Section 8.3.1(a)(ii) (DM1 Program Development Milestones) no later than [***] days after the achievement of, respectively, the DM1 First Development Milestone Event and the DM1 Second Development Milestone Event, *provided* that Sarepta has previously received an invoice from Arrowhead for such milestone payment no later than five Business Days after, as applicable, Arrowhead's issuance of its achievement notice or Arrowhead's receipt of Sarepta's achievement notice.

(c) [***]

(d) **DM1 First Development Milestone Event**. [***]

(e) **DM1 Second Development Milestone Event**. [***]

(f) [***]

(g) [***]

8.3.2. **Regulatory Milestones**. On a Program-by-Program basis, Sarepta will pay to Arrowhead one-time, non-refundable, and non-creditable milestone payments in accordance with Table 8.3.2(a), Table 8.3.2(b), and Table 8.3.2(c) below (each a "**Regulatory Milestone Payment**") upon the first achievement by Sarepta or its Affiliates or its or their Sublicensees of each of the applicable regulatory milestone events for the applicable Program as set forth in Table 8.3.2(a), Table 8.3.2(b), and Table 8.3.2(c) below (each a "**Regulatory Milestone Event**") for the first Licensed Product that is the subject of such applicable Program to achieve such applicable Regulatory Milestone Event. For the avoidance of doubt, if Sarepta or its Affiliates or their respective Sublicensees achieve all Regulatory Milestone Events with respect to (a) all Programs, then the Regulatory Milestone Payments payable by Sarepta under this Section 8.3.2 (Regulatory Milestones) will be $1,890,000,000, (b) any one of the DM1 Program, DUX4 Program, ATXN1 Program, ATXN2 Program, or ATXN3 Program, then the Regulatory Milestone Payments payable by Sarepta under this Section 8.3.2 (Regulatory Milestones) with respect to such Program will be $110,000,000, (c) any one of the MMP7 Program or HTT Program, then the Regulatory Milestone Payments payable by Sarepta under this Section 8.3.2 (Regulatory Milestones) with respect to such Program will be $130,000,000, and (d) a Category 3 Program, then the Regulatory Milestone Payments payable by Sarepta under

this Section 8.3.2 (Regulatory Milestones) with respect to such Program will be $180,000,000.

Table 8.3.2(a) –Regulatory Milestones					
Regulatory Milestone Event	***Regulatory Milestone Payment***				
	[***]	**[***]**	**[***]**	**[***]**	**[***]**
[***]	$60,000,000	$60,000,000	$60,000,000	$60,000,000	$60,000,000
[***]	$35,000,000	$35,000,000	$35,000,000	$35,000,000	$35,000,000
[***]	$15,000,000	$15,000,000	$15,000,000	$15,000,000	$15,000,000

Table 8.3.2(b) –Regulatory Milestones		
Regulatory Milestone Event	***Regulatory Milestone Payment***	
	MMP7 Program	**HTT Program**
[***]	$75,000,000	$75,000,000
[***]	$40,000,000	$40,000,000
[***]	$15,000,000	$15,000,000

Table 8.3.2(c) – Regulatory Milestones	
Regulatory Milestone Event	***Regulatory Milestone Payment***
	Each Category 3 Program
[***]	$10,000,000
[***]	$15,000,000
[***]	$25,000,000
[***]	$75,000,000
[***]	$40,000,000
[***]	$15,000,000

[***]

Sarepta will notify Arrowhead in writing of the achievement of a Regulatory Milestone Event no later than [***] days after Sarepta becomes aware of the achievement thereof, and pay to Arrowhead the corresponding Regulatory Milestone Payment no later than [***] days after the achievement of such Regulatory Milestone Event, *provided* that Sarepta has previously received an invoice from Arrowhead for such corresponding Category 2 Regulatory Milestone Payment no later than [***] Business Days after its receipt of Sarepta's achievement notice.

8.3.3. **Sales Milestones**. On a Program-by-Program basis, Sarepta will make one-time, non-refundable, and non-creditable milestone payments to Arrowhead in accordance with Table 8.3.3(a) and Table 8.3.3(b) below (each a "**Sales Milestone Payment**") upon the first achievement by Sarepta or its Affiliates or its or their Sublicensees of each of the sales milestone events set forth in Table 8.3.3(a) and Table 8.3.3(b) below for the applicable Program (each a "**Sales Milestone Event**") with respect to the aggregate annual Net Sales (subject to any reductions as set forth in Section 8.5 (Payment Reductions)) of all Licensed Products that are the subject of such applicable Program in the Territory. For the avoidance of doubt, if Sarepta or its Affiliates or their respective Sublicensees achieve all Sales Milestone Events with respect to (a) all Programs, then the Sales Milestone Payments

payable by Sarepta under this Section 8.3.2 (Regulatory Milestones) will be $8,100,000,000, (b) any one of the [***] then the Sales Milestone Payments payable by Sarepta under this Section 8.3.2 (Regulatory Milestones) for such Program will be $500,000,000, and (c) any one of the MMP7 Program, HTT Program, or a Category 3 Program, then the Regulatory Milestone Payments payable by Sarepta under this Section 8.3.2 (Regulatory Milestones) for such Program will be $700,000,000.

Table 8.3.3(a) –Sales Milestones

Sales Milestone Event	***Sales Milestone Payment***				
	[***]	**[***]**	**[***]**	**[***]**	**[***]**
[***]	$40,000,000	$40,000,000	$40,000,000	$40,000,000	$40,000,000
[***]	$60,000,000	$60,000,000	$60,000,000	$60,000,000	$60,000,000
[***]	$100,000,000	$100,000,000	$100,000,000	$100,000,000	$100,000,000
[***]	$125,000,000	$125,000,000	$125,000,000	$125,000,000	$125,000,000
[***]	$175,000,000	$175,000,000	$175,000,000	$175,000,000	$175,000,000

Table 8.3.3(b) –Sales Milestones

Sales Milestone Event	***Sales Milestone Payment***		
	MMP7 Program	**HTT Program**	**Each Category 3 Program**
[***]	$40,000,000	$40,000,000	$40,000,000
[***]	$60,000,000	$60,000,000	$60,000,000
[***]	$100,000,000	$100,000,000	$100,000,000
[***]	$125,000,000	$125,000,000	$125,000,000
[***]	$175,000,000	$175,000,000	$175,000,000
[***]	$200,000,000	$200,000,000	$200,000,000

On a Program-by-Program basis, the Net Sales of any Licensed Product that is the subject of such Program in any country in the Territory will not be included after the Royalty Term for such Licensed Product in such country has expired.

Sarepta will notify Arrowhead in writing of the achievement of a Sales Milestone Event by Sarepta or any of its Affiliates or any of its or their Sublicensees no later than [***] days after the end of the Calendar Year in which such Sales Milestone Payment is payable under this Section 8.3.3 (Sales Milestones), and pay to Arrowhead the corresponding Sales Milestone Payment no later than [***] days after the achievement of such Sales Milestone Event, *provided* that Sarepta has previously received an invoice from Arrowhead for such corresponding Sales Milestone Payment no later than five Business Days after its receipt of Sarepta's achievement notice.

For clarity, the Sales Milestone Payments will be due for each Program, but in no event will any Sales Milestone Event be due more than one time for each Program. [***]

8.4. Royalties. On a Program-by-Program basis, during the Royalty Term for each Licensed Product that is the subject of such Program in each country in the Territory and subject to the provisions of

[***] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED.

Section 8.5 (Payment Reductions), Sarepta will pay to Arrowhead nonrefundable, non-creditable royalty payments in the amount of the applicable royalty rates set forth in Table 8.4 (Royalty Payments) below based on the aggregate Net Sales resulting from the sale of [***] such Program in the Territory by Sarepta, its Affiliates, or their respective Sublicensees during each Calendar Year (such payments, "**Royalties**").

8.5. Payment Reductions.

TABLE 8.4 – Royalty Payments	
Annual Net Sales of all Licensed Products of the same Program	***Marginal Royalty Rate (% of Annual Net Sales)***
The portion of Annual Net Sales [***] less than or equal to [***]	[***]%
The portion of Annual Net Sales [***] greater than [***] and less than or equal to [***]	[***]%
The portion of Annual Net Sales [***] greater than [***]and less than or equal to [***]	[***]%
The portion of Annual Net Sales [***] greater than [***]and less than or equal to [***]	[***]%
The portion of Annual Net Sales [***] greater than [***]	[***]%

8.5.1. **Reduction for No Valid Claim**. Subject to Section 8.5.5 (Minimum Floor), on a Licensed Product-by-Licensed Product and country-by-country basis, if, within any time period during the Royalty Term for such Licensed Product in such country, such Licensed Product is not Covered by a Valid Claim of [***], the Net Sales of such Licensed Product in such country used to calculate Royalties and Sales Milestone Payments due for such Licensed Product in such country in accordance with Section 8.4 (Royalties) and Section 8.3.3 (Sales Milestones) will be reduced by [***]% during such time period.

8.5.2. **Reduction for Generic Competition**. Subject to Section 8.5.5 (Minimum Floor), on a Licensed Product-by-Licensed Product and country-by-country basis, if, during the Royalty Term for such Licensed Product in such country, one or more Generic Products with respect to such Licensed Product is approved for sale or otherwise is sold in such country in a given Calendar Quarter (the date of such first sale of a given Generic Product, such Generic Product's "**Generic Entry Date**"), then, if, in any given Calendar Quarter after the first Generic Entry Date in such country, there has been a reduction in Net Sales of such Licensed Product in such country of more than [***]% as compared to the averaged Net Sales of such Licensed Product in such country over the [***] Calendar Quarters immediately preceding the first Generic Entry Date in such country, then, commencing in the first full Calendar Quarter following such Calendar Quarter, the Net Sales of such Licensed Product in such country used to calculate Royalties due for such Licensed Product in such country in accordance with Section 8.4 (Royalties) will be reduced by [***]% [***].

8.5.3. **Third Party Payments**. Subject to Section 8.5.5 (Minimum Floor), in the event that, during the Royalty Term for a Licensed Product in a country, Sarepta makes any [***] pursuant to any agreement with a Third Party or any Platform Third Party Agreement as set forth in Section 2.9.2(b)(iii) (After Effective Date), in each case, under which Sarepta is granted rights (whether by acquisition, license, or sublicense) to any [***] in such country owned or otherwise controlled by such Third Party that are [***] for the

Development, Manufacture, or Commercialization of such Licensed Product in such country, Sarepta may credit [***]% of any [***] actually paid by Sarepta to such Third Party pursuant to the terms of such Agreement to the extent reasonably allocable to such rights in such country against any [***] payable for such Licensed Product in such country by Sarepta to Arrowhead under [***].

8.5.4. [***]

8.5.5. **Minimum Floor**. In no event will the Net Sales of a given Licensed Product in a country used to calculate (a) Royalties due and payable by Sarepta to Arrowhead under Section 8.4 (Royalties) in a given Calendar Quarter for such Licensed Product in such country be reduced to less than [***]% of the Net Sales of such Licensed Product in such country in such Calendar Quarter as a result of the aggregate reductions permitted pursuant to Section 8.5.1 (Reduction for No Valid Claim), Section 8.5.2 (Reduction for Generic Competition), Section 8.5.3 (Third Party Payments), [***].

8.6. Other Amounts Payable. With respect to any amounts owed under this Agreement by one Party to the other for which no other invoicing and payment procedure is specified in this Agreement, within [***] days after the end of each Calendar Quarter each Party will provide an invoice, together with reasonable supporting documentation, to the other Party for such amounts owed in respect of such Calendar Quarter. The owing Party will pay any undisputed amounts within [***] days after receipt of the invoice and will pay any disputed amounts owed by such Party within [***] days after resolution of the Dispute.

8.7. Payment Terms.

8.7.1. **Manner of Payment**. All payments to be made between the Parties under this Agreement will be made in Dollars and will be paid by wire transfer in immediately available funds to a bank account designated by the receiving Party; *provided* that in no event will Sarepta be obligated to make payments under this Agreement to any Affiliate of Arrowhead that is organized in any jurisdiction outside of the U.S. without Sarepta's prior written consent.

8.7.2. **Reports and Royalty Payments**. With respect to each Calendar Quarter during which Royalties are due and payable by Sarepta to Arrowhead, within [***] days after the end of such Calendar Quarter, Sarepta will submit to Arrowhead a written report including the following information listed by Licensed Product and by country or other jurisdiction of sale in the Territory: [***] and Sarepta will make any such payments within [***] days after the end of the Calendar Quarter during which the applicable Net Sales of Licensed Products occurred.

8.7.3. **Records and Audits**. Each Party will keep, and will cause its Affiliates and its Sublicensees to keep, complete, true, and accurate books and records in accordance with GAAP in relation to this Agreement, including in relation to (a) in the case of Sarepta, all Net Sales, Royalties, and Sale Milestone Payments (the "**Sarepta Records**") and (b) in the case of Arrowhead, all costs and expenses incurred in connection with the performance of Development and Manufacturing activities and any other amounts to be reimbursed by Sarepta under this Agreement (the "**Arrowhead Records**"). Each Party will keep, and will cause its Affiliates and its Sublicensees to keep, such books and records until the later of (i) [***] years following the Calendar Year to which they pertain and (ii) such period as

may be required by applicable Law. Either Party (the "**Auditing Party**") may cause an internationally-recognized independent accounting firm (the "**Auditor**") that is reasonably acceptable to the other Party (the "**Audited Party**") to inspect the relevant records of the Audited Party and its Affiliates and Sublicensees to verify the payments made by Sarepta and the related reports, statements and books of accounts, as applicable. Before beginning its audit, the Auditor will execute an undertaking reasonably acceptable to the Audited Party by which the Auditor agrees to keep confidential all information reviewed during the audit. The Audited Party and its Affiliates and Sublicensees will make their records available for inspection by the Auditor during regular business hours at such place or places where such records are customarily kept, upon receipt of reasonable advance notice from the Auditing Party. The Auditor will review such records solely to verify the accuracy of (A) in the case of Sarepta as the Audited Party, the Sarepta Records and the payments owed to Arrowhead under the financial terms of this Agreement and (B) in the case of Arrowhead as the Audited Party, the Arrowhead Records and all costs and expenses reported to have been incurred in connection with its performance of the Development and Manufacturing activities under this Agreement. Each Party will not exercise such inspection right more than once in any Calendar Year and not more frequently than once with respect to records covering any specific period of time. In addition, the Auditing Party will only be entitled to audit the books and records of the Audited Party, its Affiliates, and its Sublicensees from the three Calendar Years prior to the Calendar Year in which the Auditing Party notifies Sarepta of such audit request. Notwithstanding any provision to the contrary in Article 9 (Confidentiality and Publication), the Auditing Party agrees to hold in strict confidence all information received and all information learned in the course of any audit or inspection, except to the extent necessary for the Auditing Party to enforce its rights under this Agreement or to the extent required to comply with any applicable Law, regulation, or judicial order. The Auditor will provide its audit report and basis for any determination to the Audited Party at the time such report is provided to the Auditing Party. If the final result of the inspection reveals an undisputed underpayment or overpayment by the Audited Party, then the underpaid or overpaid amount will be settled promptly. The Auditing Party will pay for such inspections, as well as its expenses associated with enforcing its rights with respect to any payments hereunder; *provided*, *however*, that, if the final results of such audit reveal an overpayment or underpayment of more than [***]% of the total payments due hereunder for the audited period, then the fees and expenses charged by the Auditor will be paid by the Audited Party.

8.7.4. **Currency Exchange**. The rate of exchange to be used in computing the amount of currency equivalent in Dollars owed to a Party under this Agreement will be the monthly average exchange rate between each currency of origin and Dollars as reported by *The Wall Street Journal*, *East Coast Edition* or an equivalent resource as agreed by the Parties.

8.7.5. **Taxes**.

(a) **Withholding Taxes**. The amounts payable pursuant to this Agreement ("**Payments**") will not be reduced on account of any Taxes unless required by Law. The Parties acknowledge and agree that no Taxes are expected to be deducted or withheld from the Payments. If Sarepta, as a result of a change in Law after the Effective Date or otherwise as a change in circumstances is required to deduct or withhold from any Payment under any applicable Tax Law ("**Withholding Taxes**"), Sarepta will promptly (but no later than 10 Business Days prior to the

due date of the applicable Payment) notify Arrowhead in writing of the potential for Withholding Taxes and the basis therefor, and cooperate with Arrowhead in good faith so as to reduce or eliminate any potential obligation for such withholding of Taxes to the greatest extent possible, including with respect to obtaining the benefit of any present or future treaty against double Taxation or refund or reduction in such Taxes. Notwithstanding the foregoing, in the event an obligation to withhold or deduct arises as a result of an assignment, transfer, or other action by Arrowhead, the notice provision of the preceding sentence shall not apply. Subject to Section 8.7.5(c) (Assignments and Transfers), Sarepta will deduct and withhold from the Payments any Taxes that it is required by Law to deduct or withhold and will properly remit such Taxes to the appropriate Governmental Authority. Sarepta will provide Arrowhead with reasonable evidence of the proper payment of any withholding Taxes applicable to the Payments, and any receipts or certifications provided by or to a Governmental Authority, when and if available. If Withholding Taxes are paid to a Governmental Authority, then Sarepta will provide reasonable assistance to Arrowhead to obtain a refund of such Withholding Taxes, or obtain a credit with respect to Taxes paid, to the extent that such a refund or credit is available under applicable Law; *provided*, *however*, that Sarepta will be reimbursed for any reasonable Out-of-Pocket Costs.

(b) **Cooperation**. The Parties will use reasonable efforts to provide each other with information required by a Party for the purpose of filing applicable tax returns or reducing or eliminating Withholding Taxes. The Licensed Products are intended to be Exploited in the Field throughout the Territory. To the extent a Licensed Product is Exploited in the Field in a part of the Territory outside of the United States, Sarepta may cause a non-U.S. Affiliate to Commercialize such Licensed Product. In connection with this Section 8.7.5(b) (Cooperation), the Parties shall use reasonable efforts to cooperate to, as applicable, Commercialize Licensed Products in such a manner to be treated as a sale of intangible property to a recipient that is a "foreign person" (within the meaning of Treas. Reg. § 1.250(b)-4(c)) and that is for "foreign use" (within the meaning of Treas. Reg. § 1.250(b)-4(d)(2)) and either Arrowhead or a third-party advisor that is reasonably acceptable to the Parties will, at Arrowhead's sole expense, substantiate such treatment, with which Sarepta intends to reasonably cooperate. Notwithstanding anything to the contrary in this Section 8.7.5(b) (Cooperation), with respect to Sarepta's obligation to cooperate in this Section 8.7.5(b) (Cooperation), reasonable efforts will not include any actions that, in Sarepta's discretion exercised in good faith, would, or is expected to, subject Sarepta to material unreimbursed cost or expense or materially prejudice the legal, commercial, tax, or accounting position of Sarepta.

(c) **Assignments and Transfers**. If a Party that owes a Payment under this Agreement is required by Law to withhold taxes in respect of any Payment, and if such withholding obligation arises as a result of any action taken by such Party or its Affiliate or successor or assignee, including an assignment of this Agreement as permitted under Section 16.1 (Assignment) of this Agreement, a change in tax residency of such Party, or payments arise or are deemed to arise through a branch of such Party then any applicable Payments for which the recipient Party is, in the good faith discretion of the recipient Party's tax counsel or accountants (following

reasonable discussions with the paying Party or its representatives), not able to recover or credit such withheld amount in the taxable year of such payment will be increased to take into account such Withholding Taxes as may be necessary so that, after making all required Tax withholdings and deductions (including Tax withholdings and deductions on amounts payable under this Section 8.7.5 (Taxes)), the payee receives an amount equal to the sum it would have received had no such increased withholding been made.

(d) **Transfer Tax**. All transfer, documentary, sales, use, stamp, registration, and other such Taxes, and any conveyance fees, recording charges and other fees and charges (including any penalties and interest) incurred in connection with consummation of the transactions contemplated hereby, if any, will be borne and paid by Sarepta. Sarepta will prepare and timely file all tax returns required to be filed in respect of any such Taxes.

8.7.6. **Blocked Payments**. If, by reason of Law in any country, it becomes impossible or illegal for a Party to transfer, or have transferred on its behalf, any payment owed to the other Party hereunder, then such Party will (a) promptly notify the other Party of the conditions preventing such transfer and (b) deposit such payment in local currency in the relevant country to the credit of the other Party in a recognized banking institution designated by the other Party or, if none is designated by the other Party within a period of [***] days, in a recognized banking institution selected by the transferring Party, as the case may be, and identified in a written notice given to the other Party.

8.7.7. **Interest Due**. If a Party does not receive payment of any sum due to it on or before the due date, simple interest will thereafter accrue on the sum due to such Party until the date of payment at the per annum rate equal to [***].

8.7.8. **Right to Offset**. Sarepta will have the right to offset any amount (other than amounts for which Arrowhead has provided a notice of dispute with respect thereto) owed by Arrowhead to Sarepta under or in connection with this Agreement, including any amount actually owed under an indemnification obligation by Arrowhead, against any payments owed by Sarepta to Arrowhead under this Agreement. Such offsets will be in addition to any other rights or remedies available under this Agreement and applicable Law.

9. CONFIDENTIALITY AND PUBLICATION

9.1. Confidential Information. The existence and terms of this Agreement are the Confidential Information of each Party, and each Party will be deemed a Receiving Party with respect thereto. (a) Unpublished patent applications within the Licensed Product-Specific Patent Rights and Arrowhead Know-How that is specific to the composition of matter, form, formulation, or a method of treatment with, or use or manufacture of a Licensed Compound or a Licensed Product ("**Product-Specific Know-How**"), in each case, will be the Confidential Information of both Parties; (b) except as set forth in clause (e) of this Section 9.1 (Confidential Information), all Arrowhead Know-How that is neither Product-Specific Know-How nor Joint Arising Know-How will be Confidential Information of Arrowhead, *provided* that Arrowhead will maintain all Arrowhead Arising Know-How (excluding Arrowhead Excluded Know-How) in confidence and not disclose such Know-How to any Third Party for so long as such Know-How remains Confidential Information of Arrowhead, except as permitted under Section 9.2 (Non-Disclosure

and Non-Use Obligation), Section 9.4 (Permitted Disclosures), or Section 9.8 (Publication); (c) except as set forth in clause (e) of this Section 9.1 (Confidential Information), all Sarepta Arising Know-How and all reports delivered by Sarepta to Arrowhead hereunder, in each case, will be Confidential Information of Sarepta, *provided* that Sarepta will maintain all Sarepta Arising Know-How that (i) solely relates to the composition of matter, formulation, form, or a method of use or treatment, delivery, or Manufacture of a Licensed Compound or Licensed Product and (ii) is necessary or reasonably useful to Develop, Manufacture, Commercialize, and otherwise Exploit such Licensed Compound or Licensed Product, in confidence and not disclose such Know-How to any Third Party for so long as such Know-How remains Confidential Information of Sarepta, except as permitted under Section 9.2 (Non-Disclosure and Non-Use Obligation), Section 9.4 (Permitted Disclosures), or Section 9.8 (Publication); (d) all Know-How within the Joint Arising Know-How will be Confidential Information of both Parties, regardless of which Party initially generated or disclosed the relevant Joint Arising Know-How to the other Party in connection with this Agreement; and (e) all information exchanged between the Parties regarding the Prosecution and Maintenance, defense, and enforcement of the Patent Rights under Article 12 (Intellectual Property) will be the Confidential Information of both Parties. All information disclosed by a Party pursuant to the Confidentiality Agreement is deemed the Confidential Information of such Party pursuant to this Agreement.

9.2. Non-Disclosure and Non-Use Obligation. Except as otherwise expressly set forth herein, the Receiving Party will, during the Term and for a period of [***] years thereafter, keep the Confidential Information of the Disclosing Party confidential using at least the same degree of care with which the Receiving Party holds its own Confidential Information (but in no event less than a reasonable degree of care) and will not (a) disclose such Confidential Information to any Person without the prior written approval of the Disclosing Party, except, solely to the extent necessary to exercise its rights or perform its obligations under this Agreement, to its employees, Affiliates, Sublicensees, and Subcontractors, consultants, or agents who have a need to know such Confidential Information, all of whom will be similarly bound by confidentiality, non-disclosure, and non-use provisions at least as restrictive or protective of the Parties as those set forth in this Agreement and for whom the Disclosing Party will be responsible, or (b) use such Confidential Information for any purpose other than for the purposes contemplated by this Agreement. The Receiving Party will cause the foregoing Persons to comply with the restrictions on use and disclosure set forth in this Section 9.2 (Non-Disclosure and Non-Use Obligation) and will be responsible for ensuring that such Persons maintain the Disclosing Party's Confidential Information in accordance with this Article 9 (Confidentiality and Publication). Each Party will promptly notify the other Party of any misuse or unauthorized disclosure of the other Party's Confidential Information.

9.3. Exemptions. Information of a Disclosing Party will not be Confidential Information of such Disclosing Party to the extent that the Receiving Party can demonstrate through competent evidence that such information: (a) is already in the possession of the Receiving Party at the time of its receipt from the Disclosing Party and not through a prior disclosure by or on behalf of the Disclosing Party; (b) is generally available to the public before its receipt from the Disclosing Party; (c) became generally available to the public or otherwise part of the public domain after its disclosure by the Disclosing Party and other than through any act or omission of the Receiving Party or any of its Affiliates or disclosees in breach of this Agreement, including pursuant to Section 9.8 (Publications); (d) is subsequently disclosed to the Receiving Party or any of its Affiliates without obligation of confidentiality by a Third Party who may rightfully do so and is not under a conflicting obligation of confidentiality to the Disclosing Party; or (e) other than the Joint Arising Know-How

or Sarepta Arising Know-How, is developed independently by employees, Subcontractors, consultants, or agents of the Receiving Party or any of its Affiliates without use of or reliance upon the Disclosing Party's Confidential Information. No combination of features or disclosures will be deemed to fall within the foregoing exclusions merely because individual features are published or available to the general public or in the rightful possession of the Receiving Party unless the combination itself and principle of operation are published or available to the general public or in the rightful possession of the Receiving Party. Specific aspects or details of Confidential Information will not be deemed to be within the public domain or in the possession of the Receiving Party merely because the Confidential Information is encompassed by more general information in the public domain or in the possession of the Receiving Party.

9.4. Permitted Disclosures. In addition to the exceptions contained in Section 9.2 (Non-Disclosure and Non-Use Obligation), the Receiving Party may disclose Confidential Information of the Disclosing Party to the extent (and solely to the extent) that such disclosure is reasonably necessary in the following instances:

9.4.1. (a) the Prosecution and Maintenance of Patent Rights as contemplated under Article 12 (Intellectual Property); or (b) Regulatory Submissions and other filings with Governmental Authorities (including Regulatory Authorities), as necessary for the Exploitation of a Licensed Product; provided that the Receiving Party will take all reasonable measures to ensure the confidential treatment of such Confidential Information to the extent permitted under applicable Law;

9.4.2. to actual or *bona fide* potential [***], solely for the purpose of evaluating or carrying out an actual or potential [***], or [***] transaction; *provided* that, in each such case, (a) such Persons are bound by obligations of confidentiality, non-disclosure, and non-use provisions at least as restrictive or protective of the Parties as those set forth in this Agreement or otherwise customary for such type and scope of disclosure, (b) any such disclosure is limited to the maximum extent practicable for the particular context in which it is being disclosed, and (c) that the term of such confidentiality obligation must be consistent with industry standards;

9.4.3. if required by Law, including as may be required in connection with any filings made with, or by the disclosure policies of a major stock exchange (in which case the terms of such disclosures will be governed by Section 9.5 (Confidential Treatment)); *provided* that the Party seeking to disclose the Confidential Information of the other Party (other than as required by the disclosure policies of a major stock exchange): (a) use reasonable efforts to inform the other Party prior to making any such disclosures and reasonably cooperate with the other Party in seeking a protective order or other appropriate remedy (including redaction), and (b) whenever possible, request confidential treatment of such information in accordance with Section 9.5 (Confidential Treatment);

9.4.4. to prosecute or defend litigation so long as there is [***] days' prior written notice given by the Receiving Party before filing, and to enforce Patent Rights in connection with the Receiving Party's rights and obligations pursuant to this Agreement; *provided* that the Party seeking to disclose the Confidential Information of the other Party: (a) use reasonable efforts to inform the other Party prior to making any such disclosures and reasonably cooperate with the other Party in seeking a protective order or other appropriate remedy

(including redaction), and (b) whenever possible, request confidential treatment of such information in accordance with Section 9.5 (Confidential Treatment);

9.4.5. to any Third Party to the extent a Party is required to do so pursuant to the terms and conditions of an in-license agreement with such Third Party relating to the intellectual property rights sublicensed to the other Party hereunder, *provided* that any such Third Party receiving Confidential Information are bound by obligations of confidentiality, non-disclosure, and non-use provisions at least as restrictive or protective of the Parties as those set forth in this Agreement or otherwise customary for such type and scope of disclosure; and

9.4.6. with respect to Sarepta as the Receiving Party, Sarepta may use Confidential Information of Arrowhead as needed to perform its obligations and exercise its rights under this Agreement [***].

If and whenever any Confidential Information is disclosed in accordance with this Section 9.4 (Permitted Disclosures), such disclosure will not cause any such information to cease to be Confidential Information except to the extent that such disclosure results in a public disclosure of such information (other than by breach of this Agreement).

9.5. Confidential Treatment. The Parties acknowledge that either or both Parties may be obligated to file a copy of this Agreement (or portions of this Agreement or an abstract of the terms of this Agreement) with the SEC or other similar Governmental Authority in a country other than the United States. Each Party will be entitled to make such a required filing, *provided* that, it initially files a redacted copy of this Agreement (or portions of this Agreement or an abstract of the terms of this Agreement) ("**Redacted Agreement**"). In the event of any such filing, each Party will (a) permit the other Party to review and comment upon any proposed redactions of this Agreement, and request proposed redactions of this Agreement and any subsequent correspondence with respect thereto at least five Business Days in advance of its submission to the SEC or such other Governmental Authorities, (b) reasonably consider the other Party's comments thereon, and (c) if such Governmental Authority requests any changes to the redactions set forth in the Redacted Agreement, use reasonable efforts to support the redactions in the Redacted Agreement as originally filed (to the extent consistent with the then-current legal requirements governing redaction of information from material agreements that must be publicly filed) and, to the extent reasonably practicable, not agree to any changes to the redactions proposed in the Redacted Agreement without first discussing such changes with the other Party and taking the other Party's comments into consideration when deciding whether to agree to such changes. Each Party will be responsible for its own legal and other external costs in connection with any such filing, registration, or notification.

9.6. Relationship to Confidentiality Agreement. This Agreement supersedes the Confidentiality Agreement; provided, however, that all "**Confidential Information**" disclosed or received by the Parties and their Affiliates thereunder will be deemed the Confidential Information of the originally Disclosing Party hereunder and will be subject to the terms and conditions of this Agreement.

9.7. Use of Name and Logo. Subject to Section 9.8.2 (Announcements), neither Arrowhead nor Sarepta will use the other Party's or its Affiliates' name or logo in any label, press release, or product advertising, or for any other promotional purpose, without first obtaining the other Party's written consent.

[***] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED.

9.8. Publications.

9.8.1. **Coordination**. During the Term, Arrowhead and Sarepta will, from time to time and at the request of the other Party, discuss the general information content relating to this Agreement that may be publicly disclosed; *provided* that, without limitation of Arrowhead's rights under Section 9.8.3 (Publications Rights), Sarepta will have no obligation to consult with Arrowhead with respect to public announcement or publications concerning Sarepta's Exploitation of any Licensed Product that does not reference Arrowhead, or disclose any of Arrowhead's Confidential Information or the Arrowhead Platform.

9.8.2. **Announcements**. Except as may be expressly permitted under Section 9.8.1 (Coordination), Section 9.8.3 (Publications Rights), or Section 9.4 (Permitted Disclosures), during the Term, neither Party will make any public announcement regarding this Agreement without the prior written approval of the other Party, except for either Party's references to the other as the licensor or licensee (as applicable) or a collaboration partner under this Agreement. Each Party may issue a press release regarding the signing of this Agreement after the Execution Date. On or following the Execution Date, each Party will issue its respective press release in substantially the form set forth on **Schedule 9.8.2** (Press Releases). After the issuance of such press release or other permitted public disclosure by a Party, either Party may make subsequent public disclosures reiterating such information without having to obtain the other Party's prior consent and approval so long as the information in such press release or other public announcement remains true, correct, and the most current information with respect to the subject matters set forth therein. Further, Arrowhead will be permitted to issue press releases indicating the achievement of any Milestone Event and the amount of any Milestone Payment, *provided* that in all cases Arrowhead provides Sarepta with reasonable prior written notice of such press release.

9.8.3. **Publication Rights**. During the Term, Sarepta may, in its sole discretion, publish results of all Clinical Trials and other Development activities conducted with respect to any Licensed Compound or Licensed Product, *provided* that no publication will include any Confidential Information of Arrowhead, other than the Product-Specific Know-How, without Arrowhead's prior written consent, not to be unreasonably withheld, conditioned, or delayed. Arrowhead will have no such right to publish the results of Clinical Trials or other Development activities conducted with respect to any Licensed Compound or Licensed Product. Arrowhead will have the right to review all proposed publications prior to Sarepta's submission of such publication, in accordance with the procedures set forth in this Section 9.8.3 (Publications Rights). If Sarepta intends to make any publication or presentation related to any Clinical Trials or other Development activities conducted with respect to any Licensed Compound or Licensed Product, then Sarepta will first provide Arrowhead with a copy of the applicable proposed abstract, manuscript, or presentation no less than [***] days ([***] days in the case of abstracts) prior to its intended submission for publication. Arrowhead will respond in writing promptly and in no event later than [***] days ([***] days in the case of abstracts) after receipt of the proposed material with any concerns regarding the disclosure of any information or subject matter that, in Arrowhead's reasonable discretion would present issues as to patentability of the relevant subject matter or requesting the removal of any of Arrowhead's Confidential Information. In the event of any concern raised regarding protection of intellectual property rights of Arrowhead, Sarepta will not submit such publication or to make such presentation that

contains such information until Arrowhead is given a reasonable period of time, and in no event less than [***] days (or such other period as may be mutually agreed by the Parties in writing), to seek patent or other intellectual property protections in accordance with the terms of this Agreement covering any material in such publication or presentation that it believes is protectable. Subject to Section 9.4 (Permitted Disclosures), Sarepta will remove any Confidential Information of Arrowhead for which Arrowhead requests such removal from any such proposed publication or presentation. Sarepta will use reasonable efforts to provide Arrowhead with a copy of each such publication or presentation within five Business Days after the date of its submission, and in any event Sarepta will provide Arrowhead with a copy of each such publication or presentation within five Business Days after Arrowhead's written request for such copy (if not previously provided). Without limiting the foregoing, Sarepta will acknowledge the contributions of Arrowhead and the employees of Arrowhead in any such publication or presentation, as scientifically appropriate.

9.8.4. **Clinical Trial Transparency**. Both Parties agree to collaborate to maintain compliance with all Laws related to Clinical Trial transparency that may apply to either the sponsor of any Clinical Trial or the owner of any Regulatory Approval, all as related to any Licensed Product. The Parties will cooperate to maintain Clinical Trial transparency consistent with each sponsor's Clinical Trial registration, summary result, and data sharing transparency policies and will support disclosure of Confidential Information as needed based on the needs of the sponsors of the study or the Regulatory Approval holder with respect to any Licensed Product.

10. REPRESENTATIONS, WARRANTIES AND COVENANTS

10.1. Mutual Representations and Warranties. Each Party represents and warrants to the other Party, as of the Execution Date, and as of the Effective Date (as though then made), that:

10.1.1. such Party is a corporation duly organized, validly existing, and in good standing under the Laws of its jurisdiction of incorporation or formation;

10.1.2. such Party has all requisite corporate power and corporate authority to enter into this Agreement and to carry out its obligations under this Agreement;

10.1.3. all requisite corporate action on the part of such Party and its directors and stockholders required by Law for the authorization, execution, and delivery by such Party of this Agreement, and the performance of all obligations of such Party under this Agreement, has been taken;

10.1.4. the execution, delivery, and performance of this Agreement, and compliance with the provisions of this Agreement, by such Party do not and will not: (a) violate any provision of Law or any ruling, writ, injunction, order, permit, judgment, or decree of any Governmental Authority; (b) constitute a breach of, or default under (or an event that, with notice or lapse of time or both, would become a default under) or conflict with, or give rise to any right of termination, cancellation or acceleration of, any agreement, arrangement or instrument, whether written or oral, by which such Party or any of its assets are bound; or (c) violate or conflict with any of the provisions of such Party's organizational documents

(including any articles or memoranda of organization or association, charter, bylaws, or similar documents);

10.1.5. such Party has not entered into any agreement with any Third Party that is in conflict with the rights granted to the other Party under this Agreement, and has not taken any action that would prevent it from granting the rights granted to the other Party under this Agreement, or that would otherwise conflict with or adversely affect the other Party's rights under this Agreement;

10.1.6. no consent, approval, authorization, or other order of, or filing with, or notice to, any Governmental Authority or other Third Party is required to be obtained or made by such Party in connection with the authorization, execution, and delivery by such Party of this Agreement, except as required pursuant to the HSR Act and any other applicable Antitrust Laws; and

10.1.7. this Agreement has been duly executed and delivered on behalf of such Party and is a legal and valid obligation binding upon it and is enforceable in accordance with its terms, subject to applicable bankruptcy, insolvency, moratorium, and other similar laws affecting creditors' rights generally and by general principles of equity.

10.2. Additional Representations and Warranties by Arrowhead. Arrowhead represents and warrants to Sarepta, except as set forth on **Schedule 10.2** (Exceptions to the Representations and Warranties by Arrowhead), which schedule may be updated as of the Effective Date pursuant to Section 14.1 (Effective Date), as of the Execution Date and (following the Antitrust Clearance Date) as of the Effective Date (for clarity, subject to Section 14.3 (Outside Date)):

10.2.1. **Arrowhead Patent Rights**. (a) **Schedule 1.217** (Licensed Product-Specific Patent Rights) and **Schedule 1.42** (Arrowhead Platform Patent Rights) set forth a complete and accurate list of all Arrowhead Patent Rights issued or pending as of the Execution Date or the Effective Date, as applicable, and (b) the Arrowhead Patent Rights existing as of the Execution Date or the Effective Date, as applicable, constitute all of the Patent Rights owned or in-licensed by Arrowhead or any of its Affiliates as of such respective date that are necessary or reasonably useful for the Development, Manufacture, Commercialization, or other Exploitation, each as contemplated by Arrowhead or any of its Affiliates as of the Execution Date or the Effective Date, as applicable, of the Existing Lead Compounds (in each case, as they exist as of the Execution Date or the Effective Date, as applicable) in the Field in the Territory.

10.2.2. **Licensed C1 Compounds and Licensed C2 Compounds**. The Licensed C1 Compounds, Licensed C1 Products, Licensed C2 Compounds, and Licensed C2 Products include all compounds and products owned or in-licensed by Arrowhead or any of its Affiliates as of such respective date that are Directed To ATXN1, ATXN2, ATXN3, DM1, DUX4, MMP7, or HTT.

10.2.3. **Arrowhead Technology**. Arrowhead has (a) legal or beneficial title and sole ownership of, or a non-exclusive or exclusive right to use all Arrowhead Technology existing as of the Execution Date or the Effective Date, as applicable, except as set forth on **Schedule 10.2** (Exceptions to the Representations and Warranties by Arrowhead), free and clear of all mortgages, pledges, liens, encumbrances, security interests, or claims of any kind,

including claims by any Governmental Authority or academic or non-profit institution; and (b) authority to grant to Sarepta and its Affiliates the licenses set forth in Section 2.1 (License Grants to Sarepta) under the Arrowhead Technology. [***]

10.2.4. **No Conflicts**. Arrowhead has not previously assigned, transferred, conveyed, or granted any license or other rights under the Arrowhead Technology that would conflict with or limit the scope of any of the rights or licenses granted to Sarepta hereunder.

10.2.5. **Ownership of Arrowhead Technology**. With respect to all Arrowhead Technology existing as of the Execution Date or the Effective Date, as applicable, that is owned or purported to be owned by Arrowhead (a) Arrowhead and its Affiliates have obtained from all employees and independent contractors who participated in the invention or authorship thereof, assignments of all ownership rights of such employees and independent contractors in such Arrowhead Technology, either pursuant to written agreement or by operation of Law; (b) all of Arrowhead's and its Affiliates' employees, officers, contractors, and consultants have executed agreements or have existing obligations under Law requiring assignment to Arrowhead or its Affiliate, as applicable, of all rights, title, and interests in and to their inventions made during the course of and as the result of this Agreement; (c) no officer or employee of Arrowhead or any of its Affiliates is subject to any agreement with any other Third Party that requires such officer or employee to assign any interest in any Arrowhead Technology to such Third Party; and (d) Arrowhead exclusively owns all rights, title, and interests in and to the Arrowhead Patent Rights that are owned or purported to be owned by Arrowhead.

10.2.6. **Prosecution of Arrowhead Patent Rights**. The owned-Arrowhead Patent Rights, the in-licensed Arrowhead Patent Rights of which Arrowhead controls prosecution, and, to Arrowhead's knowledge, the in-licensed Arrowhead Patent Rights for which a Third Party controls prosecution, in each case, existing as of the Execution Date or the Effective Date, as applicable, are being diligently prosecuted in the respective patent offices in accordance with Law, and Arrowhead and its Affiliates have presented all references, documents, or information for which it and the inventors had a duty to disclose under Law, including 37 C.F.R. §1.56 or its foreign equivalent, to the relevant patent examiners at the relevant patent offices for each such Arrowhead Patent Right.

10.2.7. **Validity and Enforceability**. With respect to owned-Arrowhead Patent Rights, the in-licensed Arrowhead Patent Rights of which Arrowhead controls prosecution, and, to Arrowhead's knowledge, the in-licensed Arrowhead Patent Rights for which a Third Party controls prosecution, in each case, existing as of the Execution Date or the Effective Date, as applicable, there is no opposition, nullity action, interference, *inter partes* reexamination, *inter partes* review, post-grant review, derivation proceeding, or other proceeding pending or, to Arrowhead's knowledge, threatened in writing (but excluding office actions or similar communications issued by the United States Patent and Trademark Office or any analogous foreign Governmental Authority (collectively, "**Patent Offices**") in the ordinary course of Prosecution and Maintenance of any patent application) that challenge the ownership, scope, duration, validity, enforceability, or priority of any such Arrowhead Patent Right owned or purported to be owned by Arrowhead. To Arrowhead's knowledge, the Arrowhead Patent Rights that have issued are subsisting, valid, and enforceable, and Arrowhead does not have knowledge of any fact or circumstance that

would cause Arrowhead to reasonably conclude that any issued Arrowhead Patent Right is, or will be upon issuance, invalid, or unenforceable.

10.2.8. **Inventorship**. Inventorship of each owned-Arrowhead Patent Right and, to Arrowhead's knowledge, each in-licensed Arrowhead Patent Right, in each case, existing as of the Execution Date or the Effective Date, as applicable, is properly identified on each patent and patent application. To Arrowhead's knowledge, there is no dispute with respect to inventorship of any Arrowhead Patent Rights.

10.2.9. **Good Standing**. All official fees, maintenance fees, and annuities for any pending or issued owned-Arrowhead Patent Rights, in-licensed Arrowhead Patent Rights of which Arrowhead controls prosecution and maintenance, and, to Arrowhead's knowledge, in-licensed Arrowhead Patent Rights for which a Third Party controls prosecution and maintenance, in each case, existing as of the Execution Date or the Effective Date, as applicable, have been paid when due, and all administrative procedures with Governmental Authorities have been completed for such Arrowhead Patent Rights such that such Patent Rights are subsisting and in good standing.

10.2.10. **Duty of Disclosure**. To Arrowhead's knowledge, all Arrowhead Patent Rights have been duly and properly filed and maintained and the inventors thereof and parties prosecuting such applications have complied in all material respects with their duty of candor and disclosure to Patent Offices in connection with such applications.

10.2.11. **Prior Art**. To Arrowhead's knowledge, no reference or prior art would anticipate the issuance of all claims Covering each Existing Lead Compound in any patent that is an Arrowhead Patent Right pending as of the Execution Date or the Effective Date, as applicable, that is being substantively prosecuted in a non-provisional utility application.

10.2.12. **Government Funding**. No government funding, facilities of a university, college, or other educational institution or research center was used in the development of any owned-Arrowhead Patent Rights or, to Arrowhead's knowledge, in-licensed Arrowhead Patent Rights. No Person who was involved in, or who contributed to, the creation or development of any owned-Arrowhead Patent Rights or, to Arrowhead's knowledge, any in-licensed Arrowhead Patent Rights, has, performed services for the government or any university, college, or other educational institution or research center in a manner that would affect Arrowhead's rights in the Arrowhead Patent Rights.

10.2.13. **No Claims**. There is (a) no claim, judgment, or settlement against or owed by Arrowhead or any of its Affiliates and (b) no pending or, to Arrowhead's knowledge, threatened claim or litigation, in each case ((a) and (b)), related to the Arrowhead Technology or any Existing Lead Compound.

10.2.14. **Notice of Infringement or Misappropriation**. Neither Arrowhead nor any of its Affiliates have received any written notice or written threat from any Third Party asserting or alleging that any Development, Manufacture, Commercialization, or other Exploitation each as contemplated by Arrowhead or any of its Affiliates prior to the Execution Date or prior to the Effective Date, of the Existing Lead Compounds (in each case, as they exist as of the Execution Date or the Effective Date, as applicable) infringed, misappropriated, or otherwise violated any valid and enforceable Patent Right or Know-How of a Third Party.

The conception and reduction to practice of any of the Arrowhead Technology that Arrowhead purports to own have not constituted or involved the misappropriation of trade secrets of any Third Party.

10.2.15. **Third Party Technology**. To Arrowhead's knowledge, the Development, Manufacture, Commercialization, and other Exploitation, each as contemplated by Arrowhead, of any of the Existing Lead Compounds (as they exist as of the Execution Date or the Effective Date, as applicable) in the Field in the Territory does not infringe, misappropriate, or otherwise violate any valid and enforceable Patent Right or Know-How of any Third Party. [***]

10.2.16. **Third Party Infringement**. To Arrowhead's knowledge, no Third Party is infringing, misappropriating, or otherwise violating, or threatening to infringe, misappropriate, or otherwise violate the Arrowhead Technology.

10.2.17. **Confidentiality of Trade Secrets**. Arrowhead and its Affiliates have taken commercially reasonable measures consistent with industry practices to protect the secrecy, confidentiality, and value of all Arrowhead Know-How that constitutes trade secrets under Law (including requiring all employees, consultants, and independent contractors to execute binding and enforceable agreements requiring all such employees, consultants, and independent contractors to maintain the confidentiality of such Arrowhead Know-How). [***]

10.2.18. **Third Party Agreements**. Except for the Pre-Existing Third Party Agreements, there are no Third Party agreements pursuant to which Arrowhead Controls any of the Arrowhead Technology.

10.2.19. **Pre-Existing Third Party Agreements**. **Schedule 1.268** (Pre-Existing Third Party Agreements) contains a true and complete list of all agreements constituting the Pre-Existing Third Party Agreements existing as of the Execution Date or the Effective Date, as applicable, and Arrowhead has provided Sarepta with an accurate copy of each Pre-Existing Third Party Agreement. Each Pre-Existing Third Party Agreement is in full force and effect. No written notice of default or termination has been received or given under any Pre-Existing Third Party Agreement, and, to Arrowhead's knowledge, there is no act or omission by Arrowhead or any of its Affiliates that would provide a right to terminate any Pre-Existing Third Party Agreement.

10.2.20. **Compliance with Laws**. Arrowhead and its Affiliates have conducted, and, to Arrowhead's knowledge their respective contractors and consultants have conducted the Development and Manufacture of the Existing Lead Compounds in compliance with all applicable Laws, including as applicable GLP, GCP, and GMP, and any applicable anti-corruption or anti-bribery laws or regulations of any Governmental Authority with jurisdiction over such Development and Manufacture. Neither Arrowhead nor its Affiliates, nor, to Arrowhead's knowledge, any of their employees, officers, subcontractors, or consultants who have rendered services relating to the Arrowhead Technology or the Existing Lead Compounds (a) has ever been Debarred or is subject to debarment or convicted of a crime for which an entity or person could be Debarred or (b) has ever been under indictment for a crime for which a person or entity could be Debarred.

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10.2.21. **Regulatory Submissions and Study Reports**. Arrowhead or its Affiliates Control all Regulatory Submissions in the Territory for the Existing Lead Compounds existing as of the Execution Date and (following the Antitrust Clearance Date) existing as of the Effective Date, and, to Arrowhead's knowledge, Arrowhead or its Affiliates Control all study reports and underlying data from the Ongoing C1 Development Activities conducted prior to the Execution Date or (following the Antitrust Clearance Date) prior to the Effective Date. [***] and all material reports and documents required to be filed, maintained, or furnished to the FDA or any other Regulatory Authority by Arrowhead or its Affiliates for each Existing Lead Compound have been so filed, maintained, or furnished in a timely manner. [***] and all such material reports and documents were accurate and in compliance with applicable Laws, and, to Arrowhead's knowledge, were complete, in each case, on the date filed. To Arrowhead's knowledge, no event has occurred and there are no facts or circumstances reasonably likely to cause a revocation or suspension of any Regulatory Approval, or termination, seizure or suspension of the Development or Manufacture, of any Licensed Product.

10.2.22. **No Fraudulent Statements**. Neither Arrowhead nor its Affiliates, nor, to Arrowhead's knowledge, any of its or their respective directors, officers, employees or agents has (a) committed an act, (b) made a statement or (c) failed to act or make a statement, in any case ((a), (b), or (c)), that (i) would be or create an untrue statement of material fact or fraudulent statement to the FDA or any other Regulatory Authority with respect to the Development and Manufacture of any Existing Lead Compound or (ii) could reasonably be expected to provide a basis for the FDA or any other Regulatory Authority to invoke its policy respecting "Fraud, Untrue Statements of Material Facts, Bribery and Illegal Gratuities", set forth in 56 Fed. Reg. 46191 (September 10, 1991) and any amendments thereto or any analogous laws or policies, with respect to the Development or Manufacture of any Existing Lead Compound.

10.2.23. **Disclosure**. In response to any of Sarepta's requests for information in its due diligence process prior to the Execution Date, Arrowhead has not intentionally made any untrue statement of a material fact or intentionally failed to provide or otherwise disclose to Sarepta any material information known to Arrowhead or any of its Affiliates at the time of such response.

10.3. Warranty Disclaimer. EXCEPT AS OTHERWISE EXPRESSLY PROVIDED IN THIS AGREEMENT, NEITHER PARTY MAKES ANY REPRESENTATION OR EXTENDS ANY WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED, TO THE OTHER PARTY WITH RESPECT TO ANY PATENT RIGHTS, KNOW-HOW, MATERIALS, COMPOUND, PRODUCT, GOODS, SERVICES, RIGHTS OR OTHER SUBJECT MATTER OF THIS AGREEMENT AND HEREBY DISCLAIMS ALL IMPLIED WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, TITLE, OR NONINFRINGEMENT WITH RESPECT TO ANY AND ALL OF THE FOREGOING. EACH PARTY HEREBY DISCLAIMS ANY REPRESENTATION OR WARRANTY THAT THE EXPLOITATION OF ANY LICENSED COMPOUND OR LICENSED PRODUCT PURSUANT TO THIS AGREEMENT WILL BE SUCCESSFUL.

10.4. Certain Covenants.

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10.4.1. **Compliance**. Each Party and its Affiliates, Sublicensees, and Subcontractors will conduct the Exploitation of the Licensed Compounds and the Licensed Products in a good scientific manner and materially in accordance with all applicable Laws, including, as applicable, GLP, GCP, and GMP or regulations of any Governmental Authority with jurisdiction over the activities performed by or on behalf of such Party or its Affiliates, Sublicensees or Subcontractors in furtherance of such obligations. In addition, if a Party is or becomes subject to a legal obligation to a Governmental Authority (such as a corporate integrity agreement or settlement agreement with a Governmental Authority), then the other Party will perform such activities as may be reasonably requested by the obligated Party to enable such Party to comply with its legal obligation to such Governmental Authority with respect to the Licensed Products.

10.4.2. **No Debarment**. Neither Party will use or permit its Affiliates, Sublicensees, or Subcontractors to use, in any capacity in connection with the performance of its obligations under this Agreement, any Person that has been debarred pursuant to Section 306 of the FD&C Act, as amended, or that is the subject of a conviction described in such section. Each Party agrees to inform the other Party in writing immediately if it or any Person that is performing activities under this Agreement is debarred or is subject to debarment or is the subject of a conviction described in Section 306 of the FD&C Act, or if any action, suit, claim, investigation, or legal or administrative proceeding (a) has been filed and is pending or (b) is threatened in writing relating to the debarment or conviction of such notifying Party or, to such Party's knowledge, any Person or entity used in any capacity by such Party or any of its Affiliates with respect to this Agreement or the performance of its other obligations under this Agreement. Such notifying Party will use reasonable efforts to include in any agreement with any Person or entity used in any capacity by such Party or any of its Affiliates with respect to this Agreement or the performance of its other obligations under this Agreement an obligation to provide notice to such Party of the matters described in this Section 10.4.2 (No Debarment).

10.4.3. **Control**. Arrowhead or its Affiliates will retain Control during the Term of all (a) Patent Rights and Know-How owned by Arrowhead or its Affiliates as of the Effective Date that are necessary or reasonably useful to Exploit one or more Licensed Compounds or Licensed Products and (b) study reports and underlying data from Arrowhead's Development activities that are related to the Programs.

10.4.4. **No Conflicts**. During the Term, Arrowhead will not enter into any agreement with any Third Party that is in conflict with or could otherwise adversely affect the rights granted to Sarepta under this Agreement and will not take any action that would prevent it from granting the rights granted to Sarepta under this Agreement or that would otherwise materially conflict with or adversely affect the rights granted to Sarepta under this Agreement; *provided*, *however*, that the foregoing shall not prevent Arrowhead from entering into any collaboration, license, or other arrangement with a Third Party that includes Arrowhead using its platforms and other proprietary technology to Research, Develop, Manufacture, Commercialize, or otherwise Exploit compounds and products Directed To (a) any CNS Target [***] or any Cardiomyocyte Target, in each case, that is not a Collaboration Target under a Category 3 Program, (b) after the SM Exclusivity Period, any Skeletal Muscle Target (i) that is not a Collaboration Target under a Category 3 Program or (ii) so long as Sarepta has at least one Substitution Right remaining, and, in which case, solely until the date that is [***] years after the expiration of the Selection

Term, that is not a SM Reserved Target, and (c) any Collaboration Target with respect to which a Target Failure has occurred. During the Term, each Party will not, and will cause its Affiliates not to, enter into any agreement (or amend any agreement that such Party or its Affiliate is a party to as of the Effective Date) granting any license or other right in, to or under (i) such Party's interest in the Joint Arising Technology and (ii) if such Party is Sarepta, then the Sarepta Arising Technology, in each case ((i) and (ii)), that would prevent it from granting the rights granted to the other Party under this Agreement or that would otherwise conflict with or adversely affect the rights granted to the other Party under this Agreement.

10.4.5. **Export Controls**. Sarepta will not, and will ensure that its Affiliates and Sublicensees will not, export, transfer, or sell any Licensed Product (a) to any country or territory that is subject to comprehensive economic sanctions administered by OFAC, (b) to any other country or territory in which such activity would violate applicable Laws in the U.S., (c) to any Restricted Party, or (d) in such a manner that would violate the Global Trade Control Laws.

10.4.6. **No Encumbrances**. Neither Arrowhead nor any of its Affiliates will permit, nor allow to be levied, any lien, encumbrance, charge, mortgage, liability, or security interest on any Arrowhead Technology in a manner that would reasonably be expected to adversely affect the rights granted to Sarepta under this Agreement.

10.4.7. **Pre-Existing Third Party Agreements**.

(a) Arrowhead and its Affiliates will (i) not breach or be in default under any of its obligations under any Pre-Existing Third Party Agreement, in either case, in a manner that would give the applicable counterparty thereto a right to terminate such Pre-Existing Third Party Agreement (ii) will satisfy all of its obligations under each Pre-Existing Third Party Agreements, including any obligations arising due to the execution of, or activities under, this Agreement, the breach of which would give the applicable counterparty thereto a right to terminate such Pre-Existing Third Party Agreement (iii) not do any other act or make any other omission that could give rise to a termination right of any other party to any Pre-Existing Third Party Agreement, and (iv) not terminate any Pre-Existing Third Party Agreement, or amend or waive any provision thereof, in case of this clause (iv), without Sarepta's prior written consent.

(b) To the extent that the licensor in any Pre-Existing Third Party Agreement has retained any right to enforce, defend, prosecute, or maintain any Arrowhead Technology or otherwise be involved in such activities pursuant to the Pre-Existing Third Party Agreement, Arrowhead and its Affiliates will use reasonable efforts to cause such licensor to take actions (or refrain from taking action, as applicable) consistent with Article 12 (Intellectual Property).

(c) Arrowhead and its Affiliates will furnish Sarepta with copies of all material notices and material correspondences that Arrowhead or any of its Affiliates receives in connection with any Pre-Existing Third Party Agreement that are related to Sarepta's rights or obligations under this Agreement within a reasonable period following Arrowhead's or its Affiliates' receipt of the same.

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11. INDEMNIFICATION; LIMITATION OF LIABILITY; INSURANCE

11.1. Indemnification by Arrowhead. Arrowhead will indemnify, hold harmless, and defend Sarepta, its Affiliates, and their respective directors, officers, employees, and agents ("**Sarepta Indemnitees**") from and against any and all losses, liabilities, damages, costs, fees, and expenses (including reasonable attorneys' fees and litigation expenses) (collectively, "**Losses**") incurred from any claims, suits, proceedings, or causes of action brought by a Third Party (collectively, "**Claims**") against such Sarepta Indemnitees to the extent arising out of or resulting from:

11.1.1. any breach of any representation or warranty made by Arrowhead in this Agreement, or any breach or violation of any covenant or agreement of Arrowhead in this Agreement;

11.1.2. the gross negligence or willful misconduct by or of Arrowhead or any of its Affiliates, or any of their respective directors, officers, employees, or agents in the performance of Arrowhead's obligations or exercise of its rights under this Agreement; or

11.1.3. the Exploitation of any Licensed Compound or Licensed Product, in each case, by or on behalf of Arrowhead or any of its Affiliates (excluding such conduct by or on behalf of Sarepta or its Affiliates and its Sublicensees as licensees or sublicensees of Arrowhead hereunder), including the conduct of the Ongoing C1 Development Activities, the Additional R&D Activities, the Category 2 Program Research Activities, and the Category 3 Program Research Activities.

Notwithstanding the foregoing, Arrowhead will have no obligation to indemnify the Sarepta Indemnitees to the extent that the Losses arise out of or result from matters described under Section 11.2 (Indemnification by Sarepta).

11.2. Indemnification by Sarepta. Sarepta will indemnify, hold harmless, and defend Arrowhead, its Affiliates and licensees and their respective directors, officers, employees, and agents ("**Arrowhead Indemnitees**") from and against any and all Losses incurred from any Claims against such Arrowhead Indemnitees to the extent arising out of or resulting from:

11.2.1. any breach of any representation or warranty made by Sarepta in this Agreement, or any breach or violation of any covenant or agreement of Sarepta in this Agreement;

11.2.2. the gross negligence or willful misconduct by or of Sarepta or any of its Affiliates or Sublicensees, or any of their respective directors, officers, employees, or agents in the performance of Sarepta's obligations or exercise of its rights under this Agreement; or

11.2.3. the Exploitation of any Licensed Product by or on behalf of Sarepta or any of its Affiliates or Sublicensees, including the conduct of the Assumed C1 Program Development Activities.

Notwithstanding the foregoing, Sarepta will have no obligation to indemnify the Arrowhead Indemnitees to the extent that the Losses arise out of or result from matters described under Section 11.1 (Indemnification by Arrowhead).

11.3. Indemnification Procedure.

11.3.1. **Notice**. The Party entitled to indemnification under this Article 11 (Indemnification; Limitation of Liability; Insurance) (an "**Indemnified Party**") will notify the Party responsible for such indemnification (the "**Indemnifying Party**") in writing promptly (and in any event no later than ten Business Days) upon being notified of or having knowledge of any claim or claims asserted or threatened against the Indemnified Party that could give rise to a right of indemnification under this Agreement; *provided* that the failure to give such notice will not relieve the Indemnifying Party of its indemnity obligation hereunder except to the extent that such failure materially prejudices the Indemnifying Party.

11.3.2. **Indemnifying Party's Right to Defend**. Within 15 Business Days after receipt of notice from the Indemnified Party of the claim, the Indemnifying Party will have the right to defend, at its sole cost and expense and with counsel reasonably selected by the Indemnifying Party, any such claim by all appropriate proceedings and, if it elects to do so, will provide written notice of such election to the Indemnified Party within such 15-Business Day period; *provided* that the Indemnifying Party may not enter into any compromise or settlement, unless (a) such compromise or settlement (i) imposes only a monetary obligation on the Indemnifying Party and includes as an unconditional term thereof the giving by each claimant or plaintiff of the Indemnified Party a release from all liability in respect of such claim, (ii) admits no liability, wrongdoing, or other admission against interest on the part of the Indemnified Party, and (iii) would not have an adverse effect on the Indemnified Party's interests (including any rights under this Agreement or the scope or enforceability of the intellectual property licensed hereunder); or (b) the Indemnified Party consents to such compromise or settlement, which consent will not be unreasonably withheld, conditioned or delayed unless such compromise or settlement involves (i) any admission of legal wrongdoing by the Indemnified Party, (ii) any payment by the Indemnified Party that is not indemnified under this Agreement, or (iii) the imposition of any equitable relief against the Indemnified Party (in which case, (i) through (iii), the Indemnified Party may withhold its consent to such settlement in its sole discretion).

11.3.3. **Indemnified Party's Right to Defend**. If the Indemnifying Party does not elect to assume control of the defense of a claim by written notice to the Indemnified Party in accordance with Section 11.3.2 (Indemnifying Party's Right to Defend), then the Indemnified Party will have the right, at the expense of the Indemnifying Party, with written notice to the Indemnifying Party of its intent to do so, to undertake the defense of such claim for the account of the Indemnifying Party (with counsel reasonably selected by the Indemnified Party); *provided* that the Indemnified Party will keep the Indemnifying Party apprised of all material developments with respect to such claim. The Indemnified Party may not enter into any compromise or settlement without the prior written consent of the Indemnifying Party, such consent not to be unreasonably withheld, conditioned, or delayed.

11.3.4. **Cooperation**. The Indemnified Party will cooperate with the Indemnifying Party and may participate in, but not control, any defense or settlement of any claim controlled by the Indemnifying Party pursuant to this Section 11.3 (Indemnification Procedure) and will bear its own costs and expenses with respect to such participation; *provided* that the Indemnifying Party will bear such costs and expenses if counsel for the Indemnifying Party reasonably determines that such counsel may not properly represent both the Indemnifying Party and the Indemnified Party.

[***] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED.

11.4. Limitation of Liability. NEITHER PARTY WILL BE LIABLE FOR SPECIAL, INCIDENTAL, CONSEQUENTIAL, OR PUNITIVE DAMAGES ARISING OUT OF THIS AGREEMENT, OR THE EXERCISE OF ITS RIGHTS OR THE PERFORMANCE OF ITS OBLIGATIONS HEREUNDER, OR ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT, OR LOST PROFITS, REGARDLESS OF ANY NOTICE OF THE POSSIBILITY OF SUCH DAMAGES, EXCEPT FOR DAMAGES THAT ARISE AS A RESULT OF (A) A PARTY'S GROSS NEGLIGENCE OR WILLFUL MISCONDUCT, (B) A BREACH OF ARTICLE 9 (CONFIDENTIALITY AND PUBLICATION), OR (C) [***]. NOTHING IN THIS SECTION 11.4 (LIMITATION OF LIABILITY) IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER THIS AGREEMENT.

11.5. Insurance. Each Party will, at its own expense, procure and maintain during the Term and for a period of [***] years thereafter, insurance policies, including product liability insurance when applicable, adequate to cover its obligations hereunder and that are consistent with normal business practices of prudent companies similarly situated. Such insurance will not be construed to create a limit of a Party's liability with respect to its indemnification obligations under this Article 11 (Indemnification; Limitation of Liability; Insurance). Each Party will provide the other Party with written evidence of such insurance upon request. Each Party will provide the other Party with prompt written notice of cancellation, non-renewal, or material change in such insurance that could materially adversely affect the rights of such other Party hereunder and will provide such notice within [***] days after any such cancellation, non-renewal, or material change.

12. INTELLECTUAL PROPERTY

12.1. Inventions.

12.1.1. **Inventorship**. Inventorship of Arising Know-How and Arising Patent Rights will be determined in accordance with United States patent Laws.

12.1.2. **Ownership of Arising Know-How and Arising Patent Rights**.

(a) **Arrowhead**. Subject to the rights or licenses granted by Arrowhead to Sarepta under this Agreement, as between the Parties, Arrowhead will own and retain all rights, title, and interest in and to any and all: (i) (A) Arising Know-How, regardless of inventorship, that is solely related to the Delivery Ligand (the "**Arising Delivery Ligand Know-How**") and (B) Arising Know-How that is conceived, discovered, developed or otherwise made solely by or on behalf of one or more Personnel of Arrowhead (or any of its Affiliates, (sub)licensees or Subcontractors), but excluding any Arising Delivery Ligand Know-How and Joint Arising Know-How (together (i)(A) and (i)(B), the "**Arrowhead Arising Know-How**"), and (ii) (A) Arising Patent Rights, regardless of inventorship, that Cover solely any Arising Delivery Ligand Know-How (the "**Arising Delivery Ligand Patent Rights**") and (B) Arising Patent Rights that Cover solely any Arrowhead Arising Know-How set forth in the foregoing clause (i)(B) (together (ii)(A) and (ii)(B), the "**Arrowhead Arising Patent Rights**"). Sarepta hereby assigns and agrees to assign to Arrowhead all rights, title, and interest in and to any Arising Delivery Ligand Know-How that is conceived, discovered, developed, or

otherwise made by or on behalf of one or more Personnel of Sarepta (or any of its Affiliates, Sublicensees, or Subcontractors).

(b) **Sarepta**. Subject to the rights or licenses granted by Sarepta to Arrowhead under this Agreement, as between the Parties, Sarepta will own and retain all rights, title, and interest in and to any and all (i) Arising Know-How that is conceived, discovered, developed, or otherwise made solely by or on behalf of one or more Personnel of Sarepta (or any of its Affiliates, Sublicensees or Subcontractors) but excluding any Arising Delivery Ligand Know-How and Joint Arising Know-How (the "**Sarepta Arising Know-How**"), and (ii) Arising Patent Rights that Cover solely any Sarepta Arising Know-How set forth in the foregoing clause (i) (the "**Sarepta Arising Patent Rights**").

(c) **Joint**. Subject to any rights or licenses expressly granted by one Party to the other Party under this Agreement, as between the Parties, the Parties will jointly own, on an equal and undivided basis, all rights, title, and interest in and to any and all: (i) Arising Know-How that is conceived, discovered, developed, or otherwise made by or on behalf of one or more Personnel of Arrowhead (or any of its Affiliates, (sub)licensees, or Subcontractors), on the one hand, and one or more Personnel of Sarepta (or any of its Affiliates, Sublicensees, or Subcontractors), on the other hand, but excluding any Arising Delivery Ligand Know-How (the "**Joint Arising Know-How**"), and (ii) Arising Patent Rights that Cover solely any Joint Arising Know-How set forth in the foregoing clause (i) (the "**Joint Arising Patent Rights**") (collectively the Joint Arising Know-How and the Joint Arising Patent Rights, the "**Joint Arising Technology**"). Subject to the rights or licenses granted to the other Party under this Agreement, each Party will be entitled to practice, license, assign, and otherwise practice under the Joint Arising Technology without the duty of accounting or seeking consent from the other Party, and where consent is required, such consent is hereby given. Each Party, for itself and on behalf of its Affiliates, hereby assigns and agrees to assign, to the other Party an equal and undivided joint ownership interest in and to all Joint Arising Technology, to be held in accordance with this Section 12.1.2(c) (Joint).

12.1.3. **Disclosure**. Each Party will promptly disclose to the other Party all invention disclosures or other similar documents relating to Arising Know-How conceived, invented, developed, or otherwise made by or on behalf of such Party (or its Affiliates, Sublicensees (or in the case of Arrowhead (sub)licensees), or Subcontractors) hereunder during the Term that is necessary or reasonably useful to Research, Develop, Manufacture, Commercialize, or otherwise Exploit one or more Licensed Compounds or Licensed Products in the Field in the Territory, and all invention disclosures or other similar documents submitted to such Party by its or its Affiliates' employees, agents, or independent contractors relating to such Arising Know-How, and shall also respond promptly to reasonable requests from the other Party for additional information relating to such disclosures, documents, or applications.

12.1.4. **Personnel Obligations**. Each employee, agent, or independent contractor of a Party or its respective Affiliates performing work under this Agreement will, prior to commencing such work, be bound by written invention assignment obligations, including: (a) promptly reporting any invention, discovery, or other intellectual property right; (b) presently assigning to the applicable Party or Affiliate all of his or her rights, title, and interests in

and to any invention, discovery, or other intellectual property; (c) cooperating in the preparation, filing, prosecution, maintenance, and enforcement of any patent and patent application; and (d) performing all acts and signing, executing, acknowledging, and delivering any and all documents required for effecting the obligations and purposes of this Agreement. It is understood and agreed that such invention assignment agreement need not reference or be specific to this Agreement. Each Party will be solely responsible for any payments to inventors with an obligation to assign, or who do assign, their rights, title, and interests in and to any Arising Know-How and Arising Patent Rights to such Party. Arrowhead will be solely responsible for payments to inventors of any other Arrowhead Patent Rights.

12.2. Prosecution and Maintenance of Patent Rights. The Parties will conduct the Prosecution and Maintenance of the applicable Patent Rights in accordance with this Section 12.2 (Prosecution and Maintenance of Patent Rights).

12.2.1. **Sarepta Right to Prosecute Patent Rights**.

(a) On a Program-by-Program basis, as between the Parties, Sarepta will have (i) with respect to each Category 1 Program, beginning on the Effective Date, the first right (but not the obligation) to Prosecute and Maintain all Licensed Product-Specific Patent Rights, Sarepta Arising LC/LP Patent Rights, and Joint Arising Patent Rights in the Territory related to such Category 1 Program, and (ii) with respect to each Category 2 Program and each Category 3 Program, beginning on the earlier of (A) the applicable CTA Ready Package Acceptance Date for such Category 2 Program and for such Category 3 Program, respectively, and (B) the date a non-provisional patent application (including an international patent application under the PCT) for a Licensed Product-Specific Patent Right, Sarepta Arising LC/LP Patent Right, or Joint Arising Patent Right related to such Category 2 Program or Category 3 Program, as applicable, is filed, the first right (but not the obligation) to Prosecute and Maintain all Licensed Product-Specific Patent Rights, Sarepta Arising LC/LP Patent Rights, and Joint Arising Patent Rights in the Territory of such Category 2 Program or Category 3 Program, as applicable (such Patent Rights in clauses (i) and (ii), collectively, the "**Sarepta Prosecuted Patent Rights**"), using patent counsel of its choice and, with respect to the Licensed Product-Specific Patent Rights and the Joint Arising Patent Rights, reasonably acceptable to Arrowhead. Sarepta will bear all Patent Costs incurred by Sarepta for the Prosecution and Maintenance of the Sarepta Prosecuted Patent Rights. Sarepta will provide Arrowhead with material communications from any Patent Office in the Territory regarding the Sarepta Prosecuted Patent Rights, as well as a reasonable opportunity to review and comment on (1) drafts of any material filings, (2) with respect to each Category 2 Program and each Category 3 Program, drafts of any patent applications related to such Category 2 Program or such Category 3 Program, as applicable, and (3) responses to be made to such Patent Offices in advance of submitting such filings, applications, or responses. Sarepta will consider Arrowhead's comments regarding such communications and drafts in good faith. In addition, Sarepta will provide Arrowhead with copies of all such final filings, applications and responses made to any Patent Office with respect to the Sarepta Prosecuted Patent Rights in a timely manner following submission thereof. Arrowhead will (x) promptly after the Effective Date provide to Sarepta

or such patent counsel the file histories for, and correspondence with Arrowhead's existing patent counsels related to the Licensed Product-Specific Patent Rights related to the Category 1 Programs, (y) provide to Sarepta promptly after the Effective Date a report detailing the status of the Licensed Product-Specific Patent Rights related to the Category 1 Programs, and (z) provide all assistance reasonably requested by Sarepta in Sarepta's Prosecution and Maintenance of such Licensed Product-Specific Patent Rights and such Joint Arising Patent Rights (including by executing all requested documents and providing additional information with respect to the applicable Patent Rights). At its sole cost and expense, Sarepta will have the sole right to Prosecute and Maintain all Sarepta Arising Patent Rights that are not Sarepta Arising LC/LP Patent Rights.

(b) If Sarepta determines in its sole discretion to abandon or not to Prosecute and Maintain any Sarepta Prosecuted Patent Right, then Sarepta will provide Arrowhead with written notice promptly after such determination to allow Arrowhead a reasonable period of time to determine, on a country-by-country basis, in its sole discretion, its interest in assuming Prosecuting and Maintaining such Patent Right in the Territory (which notice by Sarepta will be given no later than [***] days prior to the final deadline for any pending action or response that may be due with respect to such Patent Right with the applicable Patent Office). If Arrowhead provides written notice to Sarepta expressing its interest in assuming Prosecuting and Maintaining such Patent Right, then, with respect to such Patent Right in such country in the Territory, (i) Arrowhead may, in its sole discretion and at Arrowhead's cost and expense, Prosecute and Maintain or abandon such Patent Right, and (ii) Sarepta will promptly: (A) provide to Arrowhead or counsel designated by Arrowhead the file histories for, and correspondence with existing patent counsels related to, such Patent Right; (B) provide to Arrowhead a report detailing the status of such Patent Right as of the applicable date of such notice by Sarepta; and (C) at Arrowhead's cost and expense, provide all assistance reasonably requested by Arrowhead in Arrowhead's Prosecution and Maintenance of the applicable Patent Rights (including by executing all requested documents and providing additional information with respect to the applicable Patent Rights).

12.2.2. **Arrowhead Right to Prosecute Patent Rights**.

(a) Beginning on the Effective Date, as between the Parties, Arrowhead will (i) have the first right (but not the obligation) to Prosecute and Maintain all Arrowhead Platform Patent Rights, (ii) on a Program-by-Program basis, with respect to each Category 2 Program and each Category 3 Program, until the earlier of (A) the applicable CTA Ready Package Acceptance Date for such Category 2 Program and such Category 3 Program, respectively, or (B) the date a non-provisional patent application (including an international patent application under the PCT) for a Licensed Product-Specific Patent Right or Joint Arising Patent Right of such Category 2 Program or Category 3 Program, as applicable, is filed, the first right (but not the obligation) to Prosecute and Maintain all Licensed Product-Specific Patent Rights and Joint Arising Patent Rights in the Territory related to such Category 2 Program or Category 3 Program, as applicable (such Patent Rights in clauses (i) and (ii), collectively, the "**Arrowhead Prosecuted Patent Rights**"), and (iii) the sole right (but not the obligation) to Prosecute and Maintain all Arising

Delivery Ligand Patent Rights, in each case in the Territory using outside patent counsel of its choice. Arrowhead will bear all Patent Costs incurred for the Prosecution and Maintenance of such Patent Rights. Arrowhead will keep Sarepta reasonably informed of all material matters relating to the Prosecution and Maintenance of the Arrowhead Prosecuted Patent Rights, including providing Sarepta with all material communications from any Patent Office in the Territory regarding the Arrowhead Prosecuted Patent Rights, as well as a reasonable opportunity to review and comment on (1) drafts of any material filings, (2) with respect to each Category 2 Program and each Category 3 Program, drafts of any patent applications related to such Category 2 Program or such Category 3 Program, as applicable, and (3) responses to be made to such Patent Offices in advance of submitting such filings, applications, or responses. Arrowhead will consider in good faith Sarepta's comments with respect to strategies for Prosecution and Maintenance of the Arrowhead Prosecuted Patent Rights. In addition, Arrowhead will provide Sarepta with copies of all such final filings, applications, and responses made to any Patent Office with respect to the Arrowhead Prosecuted Patent Rights in a timely manner following submission thereof. Arrowhead will provide to Sarepta promptly after the Effective Date a report detailing the status of the Arrowhead Prosecuted Patent Rights.

(b) If Arrowhead determines in its sole discretion to abandon or not to Prosecute and Maintain any Arrowhead Prosecuted Patent Right, then Arrowhead will provide Sarepta with written notice promptly after such determination with respect to the Arrowhead Prosecuted Patent Rights, Sarepta will determine, on a country-by-country basis, in its sole discretion, its interest in Prosecuting and Maintaining such Patent Right in the Territory (which notice by Arrowhead will be given no later than [***] days prior to the final deadline for any pending action or response that may be due with respect to such Patent Right with the applicable Patent Office). If Sarepta provides written notice to Arrowhead expressing its interest in Prosecuting and Maintaining such Patent Right, then, with respect to such Patent Right in such country in the Territory, (i) Sarepta may, in its sole discretion and at Sarepta's cost and expense, Prosecute and Maintain or abandon such Patent Right, and (ii) Arrowhead will promptly: (A) provide to Sarepta or counsel designated by Sarepta the file histories for, and correspondence with existing patent counsel related to, such Patent Right; (B) provide to Sarepta a report detailing the status of such Patent Right as of the applicable date of such notice by Arrowhead; and (C) at Sarepta's cost and expense, provide all assistance reasonably requested by Sarepta in Sarepta's Prosecution and Maintenance of the applicable Patent Rights (including by executing all requested documents and providing additional information with respect to the applicable Patent Rights).

12.2.3. **Cooperation**. The Parties will, and will cause their Affiliates to, cooperate and implement reasonable patent filing and prosecution strategies (including filing divisionals, continuations, or otherwise). To the extent reasonable and feasible, (a) Licensed Product-Specific Patent Rights and Arrowhead Platform Patent Rights will be pursued in mutually exclusive patent applications (which may be simultaneously filed) and in separate and distinct patent families, and (b) Sarepta Arising LC/LP Patent Rights and other Sarepta Arising Patent Rights will be pursued in mutually exclusive patent applications (which may be simultaneously filed) and in separate and distinct patent families. Further, to the extent

possible, the Parties will coordinate and determine (i) the division of Arrowhead Patent Rights as either Licensed Product-Specific Patent Right or Arrowhead Platform Patent Rights, and (ii) the division of Sarepta Arising Patent Rights as either Sarepta Arising LC/LP Patent Rights or other Sarepta Arising Patent Rights.

12.3. Third Party Infringement and Defense. The Parties will conduct the enforcement and defense of the applicable Patent Rights in accordance with this Section 12.3 (Third Party Infringement and Defense).

12.3.1. **Notices**. Each Party will promptly report in writing to the other Party any Competitive Infringement of which such Party (or any of its Affiliates or Sublicensees) becomes aware and will provide the other Party with all available evidence of such Competitive Infringement in such Party's control.

12.3.2. **Sarepta Right to Enforce**.

(a) As between the Parties, Sarepta, at its own cost and expense, will have (i) the first right, but not the obligation, to bring an appropriate suit or other action to abate any existing, alleged, or threatened Competitive Infringement involving one or more Licensed Product-Specific Patent Rights, Sarepta Arising LC/LP Patent Rights, or Joint Arising Patent Rights, and (ii) the sole right, but not the obligation, to bring an appropriate suit or other action to abate any existing, alleged, or threatened any infringement action (i) involving one or more Sarepta Arising Patent Rights that are not Sarepta Arising LC/LP Patent Rights and (ii) that is *not* a Competitive Infringement involving the Joint Arising Patent Rights.

(b) Sarepta will notify Arrowhead of its decision as to whether to take any action in accordance with Section 12.3.2(a)(i) (Sarepta Right to Enforce) at least [***] Business Days before any time limit set forth in any Law or regulation, or within [***] days after being notified of such Competitive Infringement, whichever is shorter. If Sarepta decides not to take such action with respect to a Competitive Infringement involving one or more Licensed Product-Specific Patent Rights, Sarepta Arising LC/LP Patent Rights, or Joint Arising Patent Rights, then Sarepta will so notify Arrowhead in writing, and following discussion with Sarepta and consideration in good faith of any rationale provided by Sarepta as to why Sarepta elected not to take such action, and with Sarepta's written consent (not to be unreasonably withheld, conditioned or delayed) following consideration in good faith of any rationale provided by Arrowhead, Arrowhead will have the right, but not the obligation, to commence a suit or take action to enforce the applicable Licensed Product-Specific Patent Right, Sarepta Arising LC/LP Patent Right, or Joint Arising Patent Right to abate such Competitive Infringement in the Territory, by counsel of its own choice and at its own cost and expense.

12.3.3. **Arrowhead Right to Enforce**.

(a) As between the Parties, Arrowhead, at its own cost and expense, will have (i) the first right, but not the obligation, to bring an appropriate suit or other action to abate any existing, alleged, or threatened Competitive Infringement involving the Arrowhead Platform Patent Rights or Arising Delivery Ligand Patent Rights;

provided that Arrowhead will seek and reasonably consider Sarepta's comments before determining the strategy for enforcing any Arrowhead Platform Patent Right or Arrowhead Arising Patent Right, and (ii) the sole right, but not the obligation, to bring an appropriate suit or other action to abate any existing, alleged, or threatened infringement action that is *not* a Competitive Infringement involving the Arrowhead Platform Patent Rights or the Arising Delivery Ligand Patent Rights.

(b) Arrowhead will notify Sarepta of its decision as to whether to take any action in accordance with Section 12.3.3(a)(i) (Arrowhead Right to Enforce) at least [***] Business Days before any time limit set forth in any Law or regulation, or within [***] days after being notified of such Competitive Infringement, whichever is shorter. If Arrowhead decides not to take such action with respect to any Arrowhead Platform Patent Right or Arising Delivery Ligand Patent Right, then Arrowhead will so notify Sarepta in writing, and following discussion with Arrowhead and consideration in good faith of any rationale provided by Arrowhead as to why Arrowhead elected not to take such action, and with Arrowhead's written consent (not to be unreasonably withheld, conditioned or delayed) following consideration in good faith of any rationale provided by Sarepta, Sarepta will have the right, but not the obligation, to commence a suit or take action to enforce the applicable Arrowhead Platform Patent Right or Arising Delivery Ligand Patent Right to abate such Competitive Infringement in the Territory, by counsel of its own choice and at its own cost and expense.

12.3.4. **Hatch-Waxman**. Notwithstanding any provision to the contrary set forth in this Agreement, should a Party receive a certification for a Licensed Product pursuant to the Hatch-Waxman Act, or its equivalent in a country other than the U.S., with respect to any activities under this Agreement in the Field, then such Party will promptly provide the other Party with a copy of such certification. For each Licensed Product, Sarepta will have [***] days from the date on which it receives or provides a copy of such certification to provide written notice to Arrowhead ("**H-W Suit Notice**") whether Sarepta will bring suit, at its expense, within a [***]-day period from the date of such certification. Should such [***]-day period expire without Sarepta bringing suit or providing such H-W Suit Notice, then Arrowhead will be free to bring suit in its name.

12.3.5. **Cooperation**. Each Party will provide to the Party enforcing any Patent Rights under this Section 12.3 (Third Party Infringement and Defense) reasonable assistance in such enforcement, at such enforcing Party's request and expense, including joining such action as a party plaintiff if required by Law to pursue such action or providing the enforcing Party any reasonably requested documentation or other materials. The enforcing Party will keep the other Party regularly informed of the status and progress of such enforcement efforts, including providing the other Party a reasonable opportunity to comment on the enforcing Party's determination of litigation strategy and the filing of important papers to the competent court and the enforcing Party will consider such comments in good faith.

12.3.6. **Settlement**. Neither Party will settle any claim, suit, or action that it brought under this Section 12.3 (Third Party Infringement and Defense) in a manner that would reasonably be expected to affect the other Party's rights or interests, admit fault of the other Party, or impose any monetary or other obligation on the other Party, without the prior written

consent of the other Party, which consent will not be unreasonably withheld, conditioned or delayed.

12.3.7. **Allocation of Proceeds**. Any amount recovered in any suit or other action under this Section 12.3 (Third Party Infringement and Defense), including any amount recovered in any settlement of such suit or other action, will first be used to reimburse each Party's costs and expenses with respect to such suit or other action (which reimbursement will be on a *pro rata* basis to the extent such costs and expenses exceed such recovered amount) and will thereafter be [***].

12.4. Defense. As between the Parties, the Party controlling the Prosecution and Maintenance of any Patent Right under Section 12.2 (Prosecution and Maintenance of Patent Rights), will have the right (but not the obligation), at its sole discretion and its own cost and expense, to defend against a declaratory judgment action, post-grant review proceeding, *inter partes* review, opposition proceeding, interference, or any other legal or administrative action challenging any such Patent Right. If the Party controlling such Prosecution and Maintenance of Arrowhead Platform Patent Rights, Licensed Product-Specific Patent Rights, or Sarepta Arising Patent Rights, as the case may be, under Section 12.2 (Prosecution and Maintenance of Patent Rights) does not defend such Patent Right under this Section 12.4 (Defense) within [***] days after the initiation by a Third Party of any of the foregoing actions or proceedings or such shorter period of time as is mandated by the rules of the applicable action or proceeding to commence the defense thereof, or elects not to continue any such defense (in which case it will promptly provide written notice thereof to the other Party), then the other Party will have the right (but not the obligation), at its sole discretion, to defend any such Patent Right. The defending Party will keep the other Party reasonably advised of all material developments in the conduct of any such defense. The defending Party will use reasonable efforts to provide the other Party with drafts of all material documents to be filed with the court or the applicable Patent Office and will consider in good faith all reasonable and timely comments thereto by such other Party before filing such documents. The non-defending Party will reasonably cooperate with the Party conducting the defense of such Third Party action, at such defending Party's cost and expense, including if required to conduct such defense, furnishing a power of attorney. Any awards or amounts received in defending any such action will be allocated between the Parties as provided in Section 12.3.7 (Allocation of Proceeds) applying *mutatis mutandis*.

12.5. Infringement of Third Party Rights.

12.5.1. **Notice**. If any Licensed Product becomes the subject of a Third Party's claim or assertion of infringement of a Patent Right of such Third Party within the Territory, then the Party first having notice of the claim or assertion will promptly notify the other Party.

12.5.2. **Defense**. [***] will have the first right, but not the obligation, to defend or settle any such Third Party claim or assertion of infringement of such Third Party's Patent Right, at [***] cost and expense. The non-defending Party will reasonably cooperate with the Party conducting the defense of the claim or assertion, at such defending Party's cost and expense, including if required to conduct such defense, furnishing a power of attorney. The defending Party will keep the non-defending Party reasonably advised of all material developments in the conduct of any proceedings in defending such Third Party claim or assertion. The defending Party will provide the non-defending Party with drafts of all

material papers to be filed with the court and will consider in good faith all reasonable comments thereto by the non-defending Party before filing such papers.

12.5.3. **Settlement; Licenses**. Except as otherwise provided in Article 11 (Indemnification; Limitation of Liability; Insurance), neither Party will enter into any settlement of any claim described in this Section 12.5 (Infringement of Third Party Rights) that affects the other Party's rights or interests, admits faults of the other Party, or imposes any monetary or other obligations on the other Party, without such other Party's written consent, such consent not to be unreasonably withheld, conditioned, or delayed. Each Party will have the right to decline to defend or to tender the defense of any claim described in this Section 12.5 (Infringement of Third Party Rights) upon reasonable written notice to the other Party, including if the other Party fails to agree to a settlement that the declining Party proposes. Except as otherwise provided in Article 11 (Indemnification; Limitation of Liability; Insurance), any settlement or license fees incurred by [***] under this Section 12.5.3 (Settlement; Licenses) will be allocated in accordance with the principle set forth in Section 8.5.3 (Third Party Payments) to the extent that the Patent Right that is the subject of such settlement license Covers the making, using, selling, offering for sale, or importing of a Licensed Product in the relevant country for which such rights are licensed thereunder.

12.5.4. **Other Invalidity or Unenforceability Proceedings**. If either Party desires to bring an opposition, action for declaratory judgment, nullity action, interference, declaration for non-infringement, reexamination, post-grant proceedings, or other attack upon the validity, title, or enforceability of a Patent Right owned or controlled by a Third Party and having one or more claims that Cover a Licensed Product, or the use, sale, offer for sale, or importation of a Licensed Product (*except* insofar as such action is a counterclaim to or defense of, or accompanies a defense of, a Third Party's claim or assertion of infringement under Section 12.5 (Infringement of Third Party Rights), in which case the provisions of Section 12.5 (Infringement of Third Party Rights) shall govern), such Party shall so notify the other Party and the Parties shall promptly confer to determine whether to bring such action or the manner in which to settle such action, and if any such action is brought by a Party, each Party will provide such assistance as may be reasonably requested by the other Party (at such other Party's cost) in connection with such action.

12.6. **Patent Right Extensions**. Subject to the remainder of this Section 12.6 (Patent Right Extensions), Sarepta will have the sole right to elect and file for patent term restoration or extension, supplemental protection certificate, or any of their equivalents (hereinafter, "**Patent Term Extensions**") with respect to Sarepta Prosecuted Patent Rights or other Sarepta Arising Patent Rights for any Licensed Product in the Territory, *provided* that, for the avoidance of doubt, Sarepta may not file a request for a Patent Term Extension for any Arrowhead Platform Patent Rights without Arrowhead's prior written consent, which consent shall not be unreasonably withheld, conditioned, or delayed. The Parties will discuss the strategy with respect to Patent Term Extensions and Sarepta will consider Arrowhead's comments in good faith. Upon Sarepta's request and at its cost and expense, Arrowhead will reasonably cooperate with Sarepta in any fillings made by Sarepta pursuant to this Section 12.6 (Patent Right Extensions). Sarepta will bear all Patent Costs incurred by Sarepta in making any such filing in the Territory for such Licensed Product.

12.7. **Orange Book Listing**. Sarepta and Arrowhead will discuss in good faith the Arrowhead Patent Rights or Joint Arising Patent Rights that will be included in the Orange Book maintained by the FDA or similar or equivalent patent listing or linking source, if any, in other countries in the

Territory for Licensed Products, and, after considering Arrowhead's comments in good faith, Sarepta will have the sole right to determine which Patent Rights will be included. Arrowhead will provide such assistance as may be reasonably requested by Sarepta in connection with such listing, at Sarepta's cost and expense.

12.8. **Trademarks**. Sarepta will have the right to brand Licensed Products in the Territory using Sarepta-related Trademarks and any other Trademarks it determines appropriate, which may vary by country or within a country of the Territory. Sarepta will own all rights, title, and interests in and to such Trademarks, including all goodwill associated therewith, and shall have the sole right to register and maintain such Trademarks in the countries and regions of the Territory that it determines, at Sarepta's cost and expense.

12.9. **Common Interest**. All non-public information exchanged between the Parties or between a Party's outside patent counsel and the other Party regarding the preparation, filing, prosecution, maintenance, defense and enforcement of the Arrowhead Patent Rights, Arising Patent Rights, or otherwise related to any Licensed Compound or any Licensed Product, and all shared information regarding analyses or opinions of Patent Rights or Know-How of a Third Party, will be deemed Confidential Information hereunder. The Parties agree and acknowledge that they have not waived, and nothing in this Agreement constitutes a waiver of, any legal privilege concerning any such Patent Rights, Know-How, or Confidential Information, including privilege under the common interest doctrine and similar or related doctrines. In furtherance of the foregoing, if the Parties agree that a separate agreement memorializing this understanding would be advantageous, then the Parties will negotiate and enter into a common interest agreement reflecting this understanding or any other common interest agreement as the Parties may mutually agree, including with respect to any product liability for a Licensed Product.

13. TERM AND TERMINATION

13.1. **Term**. This Agreement will commence upon the Effective Date and, if not otherwise terminated earlier pursuant to this Article 13 (Term and Termination), will continue, on a Licensed Product-by-Licensed Product and country-by-country basis, in full force and effect until the expiration of the Royalty Term applicable to such Licensed Product and such country and will expire in its entirety upon the expiration of the last Royalty Term (the "**Term**"). Upon expiration of the Royalty Term for a Licensed Product in a country in the Territory, the licenses granted by Arrowhead to Sarepta in Section 2.1.1 (Exclusive License Grants to Sarepta) with respect to such Licensed Product in such country will become fully paid, irrevocable, and perpetual.

13.2. **Termination Prior to Effective Date**. If any of the representations and warranties set forth in [***] (i) do not remain true and correct as of the Effective Date to the same extent as of the Execution Date, and (ii) would reasonably be expected to adversely affect in a material respect any of Sarepta's rights and interests hereunder, then, in either case ((a) or (b)), Sarepta may terminate this Agreement in its entirety upon written notice to Arrowhead and the Effective Date will be deemed not to have occurred.

13.3. **Termination for Convenience**.

13.3.1. **Prior to First Regulatory Approval**. Sarepta will be entitled to terminate this Agreement (a) in its entirety (for all Programs and all Licensed Compounds and all Licensed Products) throughout the Territory prior to receipt of Regulatory Approval of the first Licensed

Product that is the subject of any Program in a Major Region, (b) on a Program-by-Program basis for the entire Territory, for all Licensed Compounds and all Licensed Products that are the subject of such Program throughout the entire Territory prior to receipt of Regulatory Approval of the first Licensed Product that is the subject of such Program in a Major Region, [***] in each case [***] at its sole discretion upon 30 days' prior written notice to Arrowhead thereof.

13.3.2. **After First Regulatory Approval**. Sarepta will be entitled to terminate this Agreement (a) in its entirety (for all Programs and all Licensed Compounds and all Licensed Products) throughout the Territory after receipt of Regulatory Approval for any first Licensed Product that is the subject of any Program has been obtained in a Major Region, (b) on a Program-by-Program basis for the entire Territory, for all Licensed Compounds and all Licensed Products that are the subject of such Program throughout the entire Territory after receipt of Regulatory Approval for the first Licensed Product that is the subject of such Program has been obtained in a Major Region, [***] at its sole discretion upon [***] days' prior written notice to Arrowhead thereof.

13.3.3. [***]

13.3.4. [***]

13.4. Termination for Bankruptcy. This Agreement may be terminated in its entirety, to the extent permitted by Law, by a Party upon the filing or institution of bankruptcy, reorganization, liquidation or receivership proceedings, or upon an assignment of a substantial portion of the assets for the benefit of creditors, in each case, of the other Party (the "**Bankrupt Party**"); *provided* that in the case of any involuntary bankruptcy, reorganization, liquidation, or receivership proceeding, such right to terminate will only become effective if the Bankrupt Party consents to the involuntary bankruptcy or such proceeding is not dismissed within 60 days after the filing thereof.

13.5. Termination for Material Breach.

13.5.1. **Material Breach and Cure Period**. Subject to Section 13.5.2 (Disputes Regarding Material Breach), either Party (the "**Non-Breaching Party**") may terminate this Agreement (a) with respect to one or more Programs (for all Licensed Compounds and Licensed Products that are the subject of such Program) if the other Party (the "**Breaching Party**") has materially breached this Agreement with respect to such Program, or (b) in its entirety throughout the Territory if the Breaching Party has materially breached this Agreement in a manner that is not specific to a Program, in each case ((a) and (b)), and such material breach has not been cured within (i) 60 days after the Breaching Party's receipt of written notice from the Non-Breaching Party of such material breach if such material breach involves a failure to make a payment when due or (ii) 90 days after receipt of written notice of such breach for any other material breach by the Breaching Party from the Non-Breaching Party (such 60-day period or 90-day period, as applicable, the "**Cure Period**"). The written notice describing the alleged material breach will provide reasonably sufficient detail to put the Breaching Party on notice of such material breach. Any termination of this Agreement in its entirety or with respect to a Program pursuant to this Section 13.5.1 (Material Breach and Cure Period) will become effective at the end of the Cure Period, unless the Breaching Party has cured any such material breach prior to the expiration of such Cure Period, or, if such material breach (other than any breach involving

the failure to make a payment when due) is not curable prior to the expiration of the applicable Cure Period, then such Cure Period will be extended so long as the Breaching Party has (i) provided to the Non-Breaching Party a written plan that is reasonably calculated to effect a cure of such material breach, and (ii) the Breaching Party has commenced actions to cure such material breach during the Cure Period and commits to diligently carry out such plan as provided to the Non-Breaching Party, *provided* that, in no event will the Cure Period be extended to more than a total of 180 days.

13.5.2. **Disputes Regarding Material Breach**. If the Parties reasonably and in good faith disagree as to whether there has been a material breach or whether a material breach has been cured within the applicable Cure Period, then the Breaching Party that disputes whether there has been a material breach or cure thereof may contest the allegation in accordance with Article 15 (Dispute Resolution) and the applicable Cure Period will toll upon the initiation of such dispute resolution procedures. If, as a result of such dispute resolution process, it is finally determined pursuant to Article 15 (Dispute Resolution) that the Breaching Party committed a material breach of this Agreement, then the applicable Cure Period will resume and unless such alleged breach was cured during the pendency of such Cure Period (once resumed), this Agreement will terminate effective as of the expiration of such Cure Period. This Agreement will remain in full force and effect during the pendency of any such dispute resolution proceeding and all Cure Periods. Any such dispute resolution proceeding will not suspend any obligations of either Party hereunder, and each Party will use reasonable efforts to mitigate any damages. Any payments that are made by one Party to the other Party pursuant to this Agreement pending resolution of the Dispute will be promptly refunded if it is determined pursuant to Article 15 (Dispute Resolution) that such payments are to be refunded by one Party to the other Party. If, as a result of such dispute resolution proceeding, it is determined that the Breaching Party did not commit such material breach (or such material breach was cured in accordance with this Section 13.5 (Termination for Material Breach)), then no termination of this Agreement will be effective, and this Agreement will continue in full force and effect.

13.6. Termination for Patent Challenge. If, during the Term, Sarepta or its Sublicensee (or any Affiliate of Sarepta or any Affiliate of a Sublicensee) commences or participates in, or actively assists any other Person in bringing, any action or legal or administrative proceeding (including any patent opposition or re-examination proceeding), or otherwise asserts any claim, challenging or denying the patentability, validity, or enforceability of any claim of any Licensed Product-Specific Patent Right or Arrowhead Platform Patent Right in one or more countries (each a "**Patent Challenge**"), then Arrowhead will have the right to terminate this Agreement in its entirety upon 60 days' prior written notice to Sarepta unless Sarepta or its Sublicensee (or the applicable Affiliate of Sarepta or of such Sublicensee) causes such Patent Challenge(s) to be withdrawn within the 60-day period following receipt of written notice from Arrowhead (or in the case of *ex-parte* proceedings, multi-party proceedings, or other Patent Challenges in which Sarepta or its Sublicensee (or the applicable Affiliate of Sarepta or of such Sublicensee) does not have the power to unilaterally cause the Patent Challenge(s) to be withdrawn, Sarepta or its Sublicensee (or the applicable Affiliate of Sarepta or of such Sublicensee) withdraws as a party from such Patent Challenge(s) and ceases actively assisting any other party to such Patent Challenge(s) within such 60-day period). The foregoing sentence will not apply with respect to [***].

[***] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED.

13.7. Effects of Termination. Upon any termination of this Agreement in its entirety or in part by either Party as permitted pursuant to this Article 13 (Term and Termination), the following terms will apply with respect to this Agreement and the Terminated Products [***].

13.7.1. **Termination of Licenses**. As of the effective date of termination, all licenses granted to Sarepta under Section 2.1 (License Grants to Sarepta) with respect to the Terminated Products [***] will terminate, except that such licenses may continue solely to the extent necessary, and solely for the time periods specified in such Sections, for the prompt and diligent orderly transition or wind-down of ongoing Clinical Trials of the Terminated Products [***] under Section 13.7.4 (Ongoing Clinical Trials) or sale or other disposition of any inventory of the Terminated Products [***] as permitted under Section 13.7.13 (Sell-Off Right). Further, (a) if this Agreement is terminated in part with respect to a Program for the entire Territory, then the terms "**Licensed Products**" and "**Licensed Compounds**" will automatically be deemed to be amended to exclude, respectively, all Licensed Products and all Licensed Compounds that are the subject of such Program, [***].

13.7.2. **Exclusivity**. If this Agreement is terminated in its entirety, then the Parties' rights and obligations under Section 2.10 (Exclusivity) will terminate in their entirety. If this Agreement is terminated in part, (a) with respect to a Program [***], then the Parties' rights and obligations under Section 2.10 (Exclusivity) will terminate in the entire Territory with respect to the Target that is the subject of such Program and all Licensed Compounds and Licensed Products that are the subject of such Program, or [***] Arrowhead will have the right, independently or for or with any Third Party, to Develop, Manufacture, perform Medical Affairs, Commercialize, and otherwise Exploit in the Field in the Territory any compound or product, including any Licensed Compound or Licensed Product, that is Directed To the Target of such terminated Program. [***]

13.7.3. **Reversion License**.

(a) Sarepta, on behalf of itself and its Affiliates, hereby grants and agrees to grant (without any further subsequent action required on the part of Arrowhead) to Arrowhead and its Affiliates, an [***] license (terminable for Arrowhead's violation of the scope of such license) under the Sarepta Licensed Technology existing as of the effective date of termination hereof [***] in each case to Develop, Manufacture, Commercialize, or otherwise Exploit the applicable Terminated Products in the Field [***] that are or have been the subject of Development or Commercialization hereunder as of the effective date of termination (in the form such Terminated Products exist as of the effective date of termination), [***] (such Patent Rights and Know-How, the "**Reversion Technology**" and such license, a "**Reversion License**"). [***]

(b) Arrowhead will have the right (but not the obligation) to assume, at its cost and expense, (i) the sole responsibility for the Prosecution and Maintenance of the Sarepta Arising LC/LP Patent Rights and Joint Arising Patent Rights [***] Covering solely the Terminated Products that are included within the Reversion Technology under a Reversion License, and (ii) the sole right to take any action to enforce any such Sarepta Arising LC/LP Patent Rights and Joint Arising Patent Rights in connection with any Competitive Infringement of the Terminated Products [***].

13.7.4. **Ongoing Clinical Studies**. No later than 15 Business Days after the effective date of termination, Arrowhead will submit to Sarepta a written notice specifying with respect to all Clinical Trials of the Terminated Products [***] being conducted by or on behalf of Sarepta or its Affiliates as of the effective date of termination, (a) such Clinical Trials that are to be terminated and (b) such Clinical Trials that are to be transferred to Arrowhead or its designee. For any such Clinical Trials identified by Arrowhead in its written notice to be terminated, Sarepta will wind-down such Clinical Trials, at Sarepta's cost. For any such Clinical Trials identified by Arrowhead in its written notice to be transferred, Sarepta will transfer control to Arrowhead or its designee of such Clinical Trials and will continue to conduct such Clinical Trials after the effective date of termination, at Arrowhead's cost, for [***] (or such longer period as either Party may reasonably request and is agreed by the other Party) to enable such transfer to be completed to Arrowhead or its designee without interruption of any such Clinical Trials. In no event will Sarepta be required to enroll patients in any such Clinical Trial except as may be otherwise agreed by the Parties or as is reasonably necessary to protect patients.

13.7.5. **Transfer of Sarepta Arising Know-How**. Promptly following the effective date of termination, Sarepta will provide to Arrowhead all Sarepta Arising Know-How that is included in the Reversion Technology for the applicable Terminated Products in the Field [***] that are or have been the subject of Development or Commercialization hereunder as of the effective date of termination (in the form such Terminated Products exist as of the effective date of termination). [***]

13.7.6. **Transfer of Regulatory Submissions and Regulatory Approvals**. Promptly following the effective date of termination, in accordance with and to the extent permissible under applicable Law, Sarepta, on behalf of itself and its Affiliates and, subject to Section 13.7.9 (Sublicense Survival), Sublicensees will assign to Arrowhead or Arrowhead's designee possession and ownership of all Regulatory Submissions and Regulatory Approvals for the applicable Terminated Products [***] Controlled by such Person as of the effective date of such termination. In the event that Sarepta is unable to transfer and assign, or have transferred and assigned, to Arrowhead (or its designee) any such Regulatory Submissions or Regulatory Approvals, Sarepta, on behalf of itself and its Affiliates and, subject to Section 13.7.9 (Sublicense Survival), Sublicensees, hereby consents and grants to Arrowhead an exclusive (even as to Sarepta and its Affiliates and Sublicensees), fully-paid, royalty-free, irrevocable, perpetual, sublicensable, license and right of reference under such Regulatory Submissions and Regulatory Approvals (with the right to sublicense and grant further rights of reference) as necessary to Develop, Manufacture, Commercialize, and otherwise Exploit the applicable Terminated Products [***]; provided, however, that Sarepta and its Affiliates will retain such licenses, rights of reference or other rights under such Regulatory Submissions and Regulatory Approvals to the extent necessary to enable Sarepta or its Affiliates to perform any obligations or exercise any rights that survive such termination of this Agreement as may be expressly provided in this Agreement. [***]

13.7.7. **Continuation of Supply**. Upon Arrowhead's request, if (a) the effective date of termination is after the First Commercial Sale of any Terminated Product [***] (b) as of the effective date of such termination, Sarepta or its Affiliates or Sublicensees are Manufacturing finished product with respect to the Terminated Products for Commercialization thereof [***], and (c) as of the effective date of such termination, neither Arrowhead nor any of its Affiliates or (sub)licensees has obtained all necessary

Regulatory Approvals to Manufacture such Terminated Products [***] and procured or developed its own source of finished product supply with respect to such Terminated Products for Commercialization thereof [***] then, at Arrowhead's option and at Arrowhead's cost and expense, Sarepta or its or its Affiliates or Sublicensees will supply to Arrowhead such finished product with respect to such Terminated Products for Commercialization [***] for a period not to exceed [***] following the applicable effective date of termination at a price equal to [***].

13.7.8. **Third Party Agreements**. If Arrowhead so requests in writing, and to the extent permitted under Sarepta's obligations to Third Parties on the effective date of termination, Sarepta will assign to Arrowhead, and Arrowhead will assume, any Third Party agreements that solely relate to the Exploitation of the Terminated Products [***] to which Sarepta is a party (excluding any master agreement that could relate to the Exploitation of compounds or products other than the Terminated Products, regardless of whether such master agreement does relate to any such Terminated Product at such time); provided that (a) if the assignment of any such Third Party agreement requires the consent of any Third Party, then Sarepta will not be obligated to assign such Third Party agreement unless and until such consent is obtained (it being understood that if so requested by Arrowhead in writing, Sarepta will, at Arrowhead's cost, use reasonable efforts to obtain any such consent as promptly as reasonably practicable under the circumstances), and (b) for any Third Party agreement for which such consent is not obtained or for any excluded master agreement, Sarepta will introduce Arrowhead to the counterparty of such agreement and provide such other reasonable assistance to Arrowhead to facilitate Arrowhead's negotiation of its direct contract with such counterparty.

13.7.9. **Sublicense Survival**. Arrowhead will, at the written election of any Sublicensee (solely to the extent such Sublicensee is not then in breach of the applicable sublicense agreement and solely where such sublicense agreement was entered into by Sarepta with such Sublicensee in accordance with the terms of this Agreement) within 60 days after termination of this Agreement (or such longer period mutually agreed between Arrowhead and such Sublicensee) grant a direct license to such terminated Sublicensee, which license will not be broader in license scope, territory, or duration than such sublicense agreement granted by Sarepta to such Sublicensee and not more burdensome on Arrowhead in any material manner and no less favorable to Arrowhead than the financial terms of Article 7 (Payments).

13.7.10. **Sarepta Trademarks**. If, as of the effective date of termination, (a) Sarepta or any of its Affiliates owns any Trademarks that are used exclusively for the applicable Terminated Products [***] and (b) such Trademarks have been approved by the Regulatory Authority in a country [***] for use with such Terminated Products (such Trademarks, the "**Reversion Trademarks**"), then, at Arrowhead's written request, Sarepta, on behalf of itself and its Affiliates, (i) will assign and transfer to Arrowhead, or (ii) solely in such jurisdictions where such assignment is not permitted by applicable Law or if the assignment in the foregoing clause (i) is not effective, hereby grants (without any further subsequent action required on the part of Arrowhead, but exercisable solely upon the date of such termination of this Agreement) to Arrowhead an [***] license of, in each case ((i) and (ii)), all of Sarepta's and its Affiliates' rights, title, and interests in and to such Reversion Trademarks for the applicable country of [***], pursuant to an agreement that the Parties will negotiate and enter into after such effective date of termination, which agreement will

contain, to the extent applicable, indemnification obligations customary of such agreements applying to Arrowhead's use of such Reversion Trademarks following such assignment or license, as applicable.

13.7.11. **Return of Confidential Information**. Except in the case of Arrowhead for any Confidential Information included in the Reversion Technology that is the subject of a Reversion License, as soon as reasonably practicable after the effective date of termination, each Party, at its cost, will promptly return to the other Party(or as directed by such other Party destroy and certify to such other Party in writing as to such destruction) all of such other Party's Confidential Information that relates to the applicable Terminated Products [***] and that was provided by or on behalf of such other Party hereunder that is in the possession or control of such Party (or any of its Affiliates, Sublicensees or Subcontractors), except that such Party will have the right to retain copies of intangible Confidential Information of such other Party for legal purposes in accordance with such Party's internal compliance policies and may maintain records stored in accordance with automatic electronic archiving and back-up procedures until the ordinary course deletion thereof. Notwithstanding the return or destruction of any Confidential Information, the Parties will continue to be bound by their confidentiality obligations under this Agreement.

13.7.12. **Termination of Payment Obligations**. Except for any payment obligations under Section 13.7.13 (Sell-Off Right), as of the effective date of termination, all payment obligations hereunder with respect to the applicable Terminated Products [***] shall terminate, other than those that are accrued and unpaid as of the effective date of such termination. For clarity, notwithstanding any other provision of this Agreement, Sarepta shall remain liable to pay any Milestone Payments and Royalties to Arrowhead for any Milestone Event occurring, or deemed to have occurred in accordance with the terms of this Agreement, and Net Sales booked by Sarepta or its Affiliates or its Sublicensees, in each case, on or before the later of (a) the effective date of such termination, or (b) if applicable, [***] months following the effective date of such termination for any sales or dispositions of the applicable Terminated Product [***] that occur during such [***]-months period under Section 13.7.13 (Sell-Off Right).

13.7.13. **Sell-Off Right**. If the effective date of termination is after the First Commercial Sale of a Terminated Product [***], then, to the extent permitted by applicable Law, Sarepta and its Affiliates and Sublicensees will have the right to sell or otherwise dispose of [***], any inventory of the Terminated Products [***] for a period of [***] months following the effective date of such termination in accordance with the terms and conditions of this Agreement; *provided* that any revenue obtained from such disposal shall be treated as Net Sales and the provisions of Article 7 (Payments) shall apply to such Net Sales and, in the event that such sales result in the achievement of a Sales Milestone Event, the Sale Milestone Payment associated with such Sale Milestone Event will be owed and payable to Arrowhead. Within [***] days after the end of such [***]-month period, Sarepta will notify Arrowhead of any quantity of Terminated Products [***] remaining in Sarepta's, its Affiliates' or, subject to Section 13.7.9 (Sublicense Survival), its Sublicensees' inventory, and Arrowhead will have the right to purchase, in its discretion, any such quantities of the Licensed Products from Sarepta, its Affiliates or its Sublicensees at a supply price to be negotiated and agreed by the Parties.

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13.7.14. **IP Files Transfer**. With respect to a Terminated Product [***], (a) any Licensed Product-Specific Patent Rights Prosecuted and Maintained by Sarepta under Section 12.2.1(a) (Sarepta Right to Prosecute Patent Rights) or in respect of which Sarepta has engaged in the enforcement thereof or defense under Section 12.3.2(a) (Sarepta Right to Enforce), (b) any Arrowhead Platform Patent Rights for which Sarepta has exercised its Prosecution and Maintenance step-in rights under Section 12.2.2(b) (Arrowhead Right to Prosecute Patent Rights) or enforcement or defense step-in rights under Section 12.3.3(b) (Arrowhead Right to Enforce), and (c) if Arrowhead has elected to exercise its rights under Section 13.7.3 (Reversion License), any Sarepta Arising LC/LP Patent Rights and Joint Arising Patent Rights [***] that Cover solely the Terminated Products included within the Reversion Technology under a Reversion License, in each case ((a), (b), and (c)), at Arrowhead's cost and expense, Sarepta will transfer to Arrowhead or its designee copies of filings, applications, correspondence and other related records received or generated by Sarepta in the course of exercising any Prosecution and Maintenance activities or enforcement or defense activities.

13.7.15. **Termination of Rights and Obligations**. Except as set forth in this Section 13.7 (Effects of Termination) and Section 13.9 (Survival; Effect of Expiration or Termination), (a) as of the applicable effective date of any termination of this Agreement in its entirety all rights and obligations of the Parties under this Agreement will terminate, and (b) as of the applicable effective date of any termination of this Agreement in part, all rights and obligations of the Parties under this Agreement with respect to the terminated Program and its Terminated Products [***] will terminate.

13.8. [***]

13.9. Survival; Effect of Expiration or Termination. In addition to the termination consequences set forth in Section 13.7 (Effects of Termination) (and any Sections referenced therein), the following provisions will survive the expiration or termination of this Agreement in its entirety for any reason: (a) Article 1 (Definitions) (in each case, solely with respect to defined terms that are used in surviving provisions); (b) Section 2.9.2(b)(iii) (Platform Third Party Rights), Section 3.2.2(b) (Transition Assistance), Section 3.2.3(b) (Transition of Category 2 Program and Category 3 Research & Development Activities), Section 3.2.4 ([***]), Section 3.4.1 (Arrowhead Development Costs Reimbursement), Section 4.2.1(d) (Arrowhead Assistance), Section 5.1.1 (Category 1 Programs), Section 5.2 (Arrowhead Supply Obligation), Section 5.3 (Remaining Inventory), Section 5.4 (Manufacturing Technology Transfer), Section 8.3 (Milestone Payments), Section 8.4 (Royalties), Section 8.6 (Other Amounts Payable), Section 8.7 (Payment Terms), Section 12.3.7 (Allocation of Proceeds), and Section 8.3.1(f) ([***], in each case, solely with respect to any payment obligations that accrued prior to such expiration or termination of this Agreement but have not been paid; (c) Section 3.7 (Scientific Records) for a period of [***] years following expiration or termination or such longer period as may be required by applicable Law; (d) Section 9.7 (Use of Name and Logo), Section 10.3 (Warranty Disclaimer), Sections 11.1 (Indemnification by Arrowhead) through and including Section 11.4 (Limitation of Liability), Section 12.1 (Inventions), Section 12.9 (Common Interest), and this Section 13.9 (Survival; Effect of Expiration or Termination), Article 15 (Dispute Resolution), and Article 16 (Miscellaneous); (e) Section 9.1 (Confidential Information) through and including Section 9.6 (Relationship to Confidentiality Agreement), in each case, solely for the term specified therein; (f) Section 11.5 (Insurance) for [***] years following expiration or termination; (g) Section 12.2 (Prosecution and Maintenance of Patent Rights) solely with respect to Joint Arising Patent Rights (other than those

Joint Arising Patent Rights that Arrowhead elects to assume control of pursuant to Section 13.7.3(b) (Reversion License)); (h) Section 2.1.1 (Exclusive License Grant to Sarepta) solely in the case of expiration and not termination of this Agreement; (i) the last sentence of Section 13.1 (Term) solely in the case of expiration and not termination of this Agreement; and (j) any other provisions that, as apparent from their nature and context, are intended to continue or to remain (such as for interpretation purposes). Notwithstanding any provision to the contrary set forth in this Agreement, except as expressly set forth in Section 8.2 (Annual Fees), expiration or termination of this Agreement for any reason will not relieve the Parties of any liability or obligation that accrued hereunder prior to the effective date of such termination or expiration, nor preclude either Party from pursuing all rights and remedies it may have hereunder or at law or in equity, with respect to any breach of this Agreement.

14. EFFECTIVENESS

14.1. Effective Date . Except for the Parties' obligations under Section 3.6.1(a) (Category 1 Program Transition), Article 9 (Confidentiality and Publication), Article 10 (Representations, Warranties, and Covenants), and this Article 14 (Effectiveness), which will be effective as of the Execution Date, this Agreement will not become effective until the first Business Day after the Antitrust Clearance Date (the "**Effective Date**"); *provided* that the Effective Date will not occur if either Party exercises its termination right under Section 14.3 (Outside Date) prior to the Antitrust Clearance Date. On the Effective Date, Arrowhead will provide to Sarepta an updated version of **Schedule** 10.2 (Exceptions to the Representations and Warranties by Arrowhead) to the extent required as a result of Arrowhead making anew as of the Effective Date the representations and warranties of Section 10.2 (Additional Representations and Warranties by Arrowhead).

14.2. Filing.

14.2.1. Each Party will, within 10 Business Days following the Execution Date, file the notification and report forms required under all Antitrust Laws. The Parties will use reasonable best efforts to cooperate with one another to the extent necessary in the preparation and execution of all such documents that are required to be filed pursuant to the Antitrust Laws. Each Party will be responsible for its own costs and expenses associated with any such filing pursuant to the Antitrust Laws. The Parties will each use reasonable best efforts to ensure that any applicable waiting period under the Antitrust Laws expires or is terminated as soon as practicable and to obtain any necessary approvals or consents under any applicable Antitrust Laws, at the earliest possible date after the date of filing, and in any event prior to the one year anniversary of the Execution Date. Notwithstanding any provision to the contrary set forth in this Agreement, nothing in this Agreement (including this Section 14.2 (Filing)) will require either Party or any of its Affiliates to (a) disclose to the other Party or any of its Affiliates any information that is subject to obligations of confidentiality or non-use owed to Third Parties (nor will either Party be required to conduct joint meetings with any Governmental Authority in which such information is intended to be disclosed) in connection with any Antitrust Filing, (b) commit to any consent decree or similar undertaking, or any divestiture, license (in whole or in part), or any arrangement to hold separate (or any similar arrangement) with respect to any of its products or assets, or (c) litigate. If a Party receives a request for information or documentary material from any Governmental Authority with respect to this Agreement or the transactions contemplated hereby, including a Second Request for Information under the HSR Act, then such party shall in good faith make, or cause to be made, as soon as

reasonably practicable and after consultation with the other party, a response that is, at a minimum, in substantial compliance with such request.

14.2.2. In furtherance of the foregoing, each Party shall consult and cooperate with the other Party, including: (a) promptly notify the other Party of, and if in writing, furnish the other Party with copies of, any communications from or with any Governmental Authority with respect to this Agreement; (b) permit the other Party to review and discuss in advance, and consider in good faith the view of the other in connection with, any proposed substantive written or oral communication with any Governmental Authority; (c) not participate in any substantive meeting or have any substantive communication with any Governmental Authority unless it has given the other Party a reasonable opportunity to consult with it in advance and, to the extent permitted by such Governmental Authority, gives the other Party the opportunity to attend; (d) furnish the other Party's outside legal counsel with copies of all filings and communications between it and any such Governmental Authority with respect to this Agreement; provided, however, that such material may be redacted as necessary to (i) comply with contractual arrangements, (ii) address legal privilege concerns, and (iii) comply with Law; and (e) furnish the other Party's outside legal counsel with such necessary information and reasonable assistance as the other Party's outside legal counsel may reasonably request in connection with its preparation of submissions of information to any such Governmental Authority. The Parties may, as they deem advisable and necessary, designate any competitively sensitive materials provided to the other Party under this Section 14.2 (Filing) as "outside counsel only." Such materials and the information contained therein shall be given only to outside counsel and outside economic consultants of the recipient and will not be disclosed by such outside counsel or outside economic consultants to employees, officers, or directors of the recipient without the advance written consent of the Party providing such materials. Notwithstanding anything to the contrary in this Section 14.2 (Filing), materials provided to the other Party or its outside legal counsel may be redacted to remove references concerning the valuation of the Licensed Compounds or Licensed Products.

14.3. **Outside Date**. This Agreement will terminate at the election of either Party, immediately upon written notice by such Party to the other Party, in the event that the Antitrust Clearance Date has not occurred on or prior to the one year anniversary of the Execution Date and the Parties have not agreed in writing to extend the Antitrust Clearance Date. In the event of such termination, this Agreement will be of no further force and effect.

15. DISPUTE RESOLUTION

15.1. **Exclusive Dispute Resolution Mechanism**. The Parties agree that, except as expressly set forth in Section 7.5 (Resolution of Committee Disputes), the procedures set forth in this Article 15 (Dispute Resolution) will be the exclusive mechanism for resolving any dispute, controversy, or claim between the Parties arising out of or relating to this Agreement (whether based on contract, tort or otherwise) (each, a "**Dispute**," and collectively, the "**Disputes**") that is not resolved through good faith negotiation between the Parties pursuant to Section 15.2 (Resolution by Executive Officers). For the avoidance of doubt, this Article 15 (Dispute Resolution) will not apply with respect to any decision under the purview of the JSC, for which final decision-making authority is set forth in Section 7.5 (Resolution of Committee Disputes).

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15.2. Resolution by Executive Officers. Except as expressly set forth in Section 7.5 (Resolution of Committee Disputes) or as provided in Section 15.5 (Preliminary Injunctions), in the event of any Dispute regarding the construction or interpretation of this Agreement, or any right, obligation, or liability of either Party hereunder, the Parties will first attempt in good faith to resolve such Dispute by negotiation and consultation between themselves. In the event that such Dispute is not resolved on an informal basis within [***] Business Days, either Party may, by written notice to the other Party, refer the Dispute to the Executive Officers of the Parties for attempted resolution by good faith negotiation within [***] days after such notice is received. If the Executive Officers cannot resolve the Dispute within such [***]-day period, then (a) if the Dispute has been expressly stated in this Agreement to be resolved pursuant to expedited arbitration, it will be resolved in accordance with Section 15.3 (Expedited Arbitration), or (b) with respect to any other Dispute, either Party will have the right to pursue any and all remedies available at law or equity consistent with Section 15.4 (Litigation), *provided*, *however*, that any Dispute with respect to (i) the scope, construction, validity, or enforceability of any Patent Right or Trademark relating to a Licensed Product will be resolved by litigation in accordance with Section 15.6 (Patent and Trademark Disputes) or (ii) any antitrust, anti-monopoly, or competition law or regulation, whether or not statutory may be submitted in any court of competent jurisdiction over such Dispute.

15.3. Expedited Arbitration. Any Dispute remaining unresolved after escalation to the Parties' respective Executive Officers in accordance with Section 15.2 (Resolution by Executive Officers) or expressly stated in this Agreement to be resolved pursuant to expedited arbitration will be resolved pursuant to the following procedures of this Section 15.3 (Expedited Arbitration).

15.3.1. For purposes of arbitration under this Section 15.3 (Expedited Arbitration), the arbitration will be administered by JAMS pursuant to its rules then in effect at the time of submission for such proceedings, as modified by this Section 15.3 (Expedited Arbitration). The arbitration will be governed by the Laws of the State of New York, without giving effect to any choice of law principles that would require the application of the laws of a different state or jurisdiction. The arbitration will be heard and determined by a single arbitrator appointed by agreement of the Parties or, failing such mutual agreement, by JAMS, and who will be a single independent, conflict-free arbitrator having the requisite pharmaceutical and biotechnology industry experience (such arbitrator, the "**Arbitrator**"). The Parties may select a different Arbitrator for each Dispute depending on the nature of the issues presented and desired expertise. The arbitration will be conducted as a "baseball" form of binding arbitration conducted by the Arbitrator.

15.3.2. No later than [***] Business Days after the Arbitrator's appointment, each Party will submit to both the Arbitrator and the other Party a detailed written proposal setting forth its proposed resolution of such Dispute. The Parties will also provide to the Arbitrator a copy of this Agreement, as may have been amended at such time in accordance with Section 16.4 (Entire Agreement; Amendments).

15.3.3. No later than five Business Days after the delivery of the Parties' detailed written proposals to the Arbitrator, each Party will submit to both the Arbitrator and the other Party a legal brief (and any exhibits) explaining and supporting the Party's detailed written proposal, which legal brief will be no more than 30 pages.

15.3.4. There will be no discovery and there will be no hearing, although such arbitration proceeding will be deemed to have its seat in New York, New York, and all arbitration proceedings will be conducted in the English language.

15.3.5. No later than [***] Business Days after the submission of the Parties' legal briefs, the Arbitrator will select one of the two detailed written proposals (without modification) provided by the Parties that the Arbitrator believes is most consistent with the intention underlying the agreed principles set forth in this Agreement. The decision of the Arbitrator will be final and unappealable. The detailed written proposal selected by the Arbitrator will automatically be binding on the Parties.

15.3.6. The Arbitrator will select one of the two detailed written proposals and may not combine elements of both detailed written proposals or make any other modifications to the selected detailed written proposal.

15.3.7. Each Party will bear its own attorneys' fees, costs, and disbursements arising out of the arbitration, and will pay an equal share of the fees and costs of the Arbitrator.

15.4. Litigation. Except for Disputes expressly specified in this Agreement to be resolved pursuant to Section 15.3 (Expedited Arbitration) or Section 15.6 (Patent and Trademark Disputes), unless otherwise prohibited by applicable Law, any unresolved Dispute that was subject to Section 15.2 (Resolution by Executive Officers) will be brought exclusively in the state courts of the State of New York in the Borough of Manhattan or the federal courts in the United States District Court for the Southern District of New York, and in no other jurisdiction. Each Party hereby irrevocably consents to personal jurisdiction and venue in, and irrevocably agrees to service of process issued or authorized by any such court in any such action or proceeding. The Parties hereby irrevocably waive any objection that they may now have or hereafter have to the laying of venue in the state courts of the State of New York in the Borough of Manhattan or the federal courts in the United States District Court for the Southern District of New York in any such action or proceeding, and hereby irrevocably waive and agree not to plead or claim in any such court that any such action or proceeding brought in any such court has been brought in an inconvenient forum. The Parties hereby agree that any final judgment rendered by any such federal or state court of New York in any action or proceeding involving any Dispute, from which no appeal can be or is taken, may be enforced by the prevailing Party in any court of competent jurisdiction.

15.5. Equitable Relief. The Parties agree that monetary damages may not be a sufficient remedy for any breach of this Agreement. Notwithstanding any provision to the contrary set forth in this Agreement, in the event of an actual or threatened breach of a Party's obligations under this Agreement, a Party may seek a temporary restraining order, preliminary injunction, or other equitable relief from any court of competent jurisdiction in order to prevent immediate and irreparable injury, loss, or damage on a provisional basis.

15.6. Patent and Trademark Disputes. Notwithstanding any provision to the contrary set forth in this Agreement, any and all issues regarding the scope, construction, validity, and enforceability of any Patent Rights or Trademark relating to a Licensed Compound or Licensed Product that is the subject of this Agreement will be determined in a court or other tribunal, as the case may be, of competent jurisdiction under the applicable patent or trademark laws of the country in which such Patent Rights or Trademark rights were granted or arose.

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15.7. Payment Tolling. During the pendency of any Dispute resolution proceeding between the Parties under this Article 15 (Dispute Resolution) regarding the obligation to make any payment under this Agreement from one Party to the other Party (in whole or in part), the obligation to make such payment will be tolled until the final outcome of such Dispute has been established.

15.8. Confidentiality. Any and all activities conducted under this Article 15 (Dispute Resolution), including any and all proceedings and decisions hereunder, will be deemed Confidential Information of each of the Parties, and will be subject to Article 9 (Confidentiality and Publication) to the extent applicable in accordance with Law.

16. MISCELLANEOUS

16.1. Assignment.

16.1.1. **General**. Neither Party may assign or transfer this Agreement or any rights or obligations hereunder without the prior written consent of the other Party, *except* that a Party may make such an assignment without the other Party's consent to (a) an Affiliate pursuant to Section 16.13 (Performance by Affiliates), *provided* that such assigning Party will remain responsible for such Affiliate's conduct and compliance with its obligations under this Agreement, or (b) a Third Party as a successor to all or substantially all of the business of such Party to which this Agreement relates, whether in a merger, sale of stock, acquisition, sale of assets or similar transaction or series of related transactions, *provided* that such transaction is not primarily for the benefit of such Party's creditors, [***]. Any successor or assignee of any right or obligation permitted hereunder will, in writing to the other Party, expressly assume performance of such right or obligation. Any permitted assignment will be binding on the successors of the assigning Party. Any assignment or attempted assignment by either Party in violation of the terms of this Section 16.1.1 (General) will be null, void, and of no legal effect.

16.1.2. **Securitization Transaction**. Notwithstanding any provision to the contrary in Section 16.1.1 (General) or elsewhere in this Agreement, Arrowhead may assign to a Third Party its right to receive the Milestone Payments and the Royalties (such assignment, a "**Securitization Transaction**"). In connection with a contemplated Securitization Transaction and after the closing of any such Securitization Transaction, Arrowhead may disclose to such Third Party the royalty reports contemplated under Section 8.7.2 (Reports and Royalty Payments), without the prior written consent of Sarepta, to the extent reasonably necessary to enable such Third Party to evaluate the Securitization Transaction opportunity (*provided* that such Third Party is under obligations of confidentiality and non-use with respect to Confidential Information included in such reports and plans that are no less protective or restrictive than the terms of Article 9 (Confidentiality and Publication) (but of duration customary in confidentiality agreements entered into for a similar purpose)), and to enable such Third Party to exercise its rights with respect to such Securitization Transaction, as applicable. As part of any consummated Securitization Transaction, subject to the terms of this Section 16.1.2 (Securitization Transaction), Arrowhead may assign, without the prior written consent of Sarepta, its right to receive the royalty reports and to conduct audits under, respectively, Section 8.7.2 (Reports and Royalty Payments) and Section 8.7.3 (Records and Audits) to the counterparty in such Securitization Transaction, and to allow such counterparty to exercise its rights under such Sections. Arrowhead agrees to provide written notice to Sarepta of any process run by or

on behalf of Arrowhead involving a Securitization Transaction and to negotiate in good faith with Sarepta should Sarepta elect to submit a bid for such Securitization Transaction, *provided* that Arrowhead will in no way be precluded from soliciting other bids and conducting contemporaneous negotiations with other Third Party bidders for such Securitization Transaction.

16.2. Section 365(n) of the Bankruptcy Code. All rights and licenses now or hereafter granted under or pursuant to this Agreement by a Party to the other are and will otherwise be deemed to be, for purposes of Section 365(n) of the Bankruptcy Code, a license of a right to "intellectual property" as defined in the Bankruptcy Code. Upon the filing or institution of bankruptcy, reorganization, liquidation, or receivership proceedings, upon the appointment of a receiver or trustee over all or substantially all property, or upon an assignment of a substantial portion of the assets for the benefit of creditors by a Party, such Party agrees that the other Party, as licensee of such rights under this Agreement, will retain and may fully exercise all of its rights and elections under the Bankruptcy Code. Subject to Section 365 of the Bankruptcy Code, each Party will, during the Term, create and maintain current copies or, if not amenable to copying, other appropriate embodiments, to the extent feasible, of all intellectual property rights licensed under this Agreement. Each Party acknowledges and agrees that "embodiments" of intellectual property rights within the meaning of Section 365(n) include laboratory notebooks, cell lines, product samples, and inventory, research studies and data, all Regulatory Approvals (and all applications for Regulatory Approval) and rights of reference therein, in each case, to the extent licensed by a Party to the other Party hereunder, as well as the Arrowhead Technology and the Sarepta Licensed Technology (as the case may be), and all information related to the Arrowhead Technology and the Sarepta Licensed Technology (as the case may be). If (a) a case under the Bankruptcy Code is commenced by or against the debtor Party, (b) this Agreement is rejected as provided in the Bankruptcy Code and (c) the non-debtor Party elects to retain its rights hereunder as provided in Section 365(n) of the Bankruptcy Code and upon written request of the non-debtor Party, then:

16.2.1. the non-debtor Party will be authorized to retain and exercise its rights under this Agreement (including a right to enforce any exclusivity provision contained herein) to intellectual property rights (including all embodiments thereof to the extent protected by applicable non-bankruptcy law) licensed hereunder and held by the debtor Party as such rights existed immediately before the commencement of the case referenced in Section 13.4 (Termination for Bankruptcy), subject to the provisions of Section 365(n) of the Bankruptcy Code related to, among other things, payment of the royalties and waiver of rights to setoff and any claim allowable under Section 503(b) of the Bankruptcy Code related to the performance of this Agreement, but neither such provision nor such performance by the non-debtor Party will release the debtor Party from liability resulting from rejection of the license or the failure to perform such obligations;

16.2.2. to the extent provided herein, the debtor Party will provide to the non-debtor Party any intellectual property (including any applicable embodiment) held by the debtor Party; and

16.2.3. the debtor Party will not interfere with the non-debtor Party's rights under this Agreement, or any agreement supplemental hereto, with respect to such intellectual property rights (including such embodiments), including any right to obtain such intellectual property rights (or such embodiments) from another entity, to the extent provided in Section 365(n) of the Bankruptcy Code.

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16.3. **Governing Law**. This Agreement was prepared in the English language, which language will govern the interpretation of, and any Dispute regarding, the terms of this Agreement. This Agreement and all Disputes arising out of or related to this Agreement or any breach hereof will be governed by and construed under the laws of the State of New York, without giving effect to any choice of law principles that would require the application of the laws of a different state or jurisdiction. Notwithstanding any other provision in this Agreement, the Parties expressly reject the application to this Agreement, all transactions and activities contemplated hereby, and all Disputes of (a) the United Nations Convention on Contracts for the International Sale Of Goods, and (b) the 1974 Convention on the Limitation Period in the International Sale of Goods, as amended by that certain Protocol, concluded at Vienna, Austria on April 11, 1980.

16.4. **Entire Agreement; Amendments**. This Agreement, including the Exhibits and Schedules hereto, set forth the complete, final, and exclusive agreement and all the covenants, promises, agreements, warranties, representations, conditions, and understandings between the Parties hereto with respect to the subject matter hereof and supersedes, as of the Effective Date, all prior and contemporaneous agreements and understandings between the Parties with respect to the subject matter hereof. There are no covenants, promises, agreements, warranties, representations, conditions, or understandings, either oral or written, between the Parties other than as are set forth herein and therein. No subsequent alteration, amendment, change, or addition to this Agreement will be binding upon the Parties unless reduced to a writing explicitly stating the Parties' intent to amend this Agreement that is signed by an authorized officer of each Party. If there is any inconsistency between the body of this Agreement and either any Exhibits or Schedules to this Agreement or any subsequent agreements ancillary to this Agreement, then, unless otherwise express stated to the contrary in such Exhibit, Schedule or ancillary agreement, the terms contained in this Agreement will control.

16.5. **Severability**. If any one or more of the provisions of this Agreement is held to be invalid, illegal or unenforceable by any court or tribunal of competent jurisdiction from which no appeal can be or is taken, then the provision will be considered severed from this Agreement and will not serve to invalidate any remaining provisions hereof. The Parties will make a good faith effort to replace any invalid or unenforceable provision with a valid and enforceable one such that the objectives contemplated by the Parties when entering this Agreement may be realized.

16.6. **Headings**. The captions to the Sections hereof are not a part of this Agreement, but are merely for convenience to assist in locating and reading the several Sections hereof.

16.7. **Interpretation**. Except where the context otherwise requires, wherever used, the singular will include the plural, the plural will include the singular, and the use of any gender will be applicable to all genders. Whenever this Agreement refers to a number of days without using a term otherwise defined herein, such number refers to calendar days. The captions of this Agreement are for the convenience of reference only and in no way define, describe, extend, or limit the scope or intent of this Agreement or the intent of any provision contained in this Agreement. The terms "including," "include," "includes," or "for example" will not limit the generality of any description preceding such term and as used herein will have the same meaning as "including, but not limited to" or "including, without limitation." The word "will" will be construed to have the same meaning and effect as the word "shall." References to any specific law, rule or regulation, or article, section or other division thereof, will be deemed to include the then-current amendments thereto or any replacement or successor law, rule or regulation thereof. The term "or" will be interpreted in the inclusive sense commonly associated with the term "and/or." Any reference herein to any person or entity will be construed to include the person's or entity's successors and assigns. The words

"herein," "hereof," and "hereunder", and words of similar import, will be construed to refer to this Agreement in its entirety and not any particular provision. The word "notice" means notice in writing (whether or not specifically stated) and will include notices, consents, approvals, and other written communications contemplated under this Agreement. References to "**Section**" or "**Sections**" are references to the numbered sections of this Agreement, unless expressly stated otherwise. All dollars are US Dollars. Unless the context otherwise requires, countries will include territories. Except as otherwise expressly set forth in this Agreement, when applied to Sarepta, the phrases "at its own cost and expense," "at its sole cost and expense," "at its cost and expense," and similar phrases used in this Agreement do not preclude the possibility that Sarepta may share such costs or expenses with a Third Party. The language of this Agreement will be deemed to be the language chosen by the Parties and no rule of strict construction will be applied against either Party hereto. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

16.8. **Waiver and Non-Exclusion of Remedies**. Any term or condition of this Agreement may be waived at any time by the Party that is entitled to the benefit thereof, but no such waiver will be effective unless set forth in a written instrument duly executed by or on behalf of such Party waiving such term or condition. The waiver by either Party of any right hereunder or of the failure to perform or of a breach by the other Party will not be deemed a waiver of any other right hereunder or of any other breach or failure by such other Party whether of a similar nature or otherwise. The rights and remedies provided herein are cumulative and do not exclude any other right or remedy provided by Law or otherwise available except as expressly set forth herein.

16.9. **Notices**. All notices and other communications given or made pursuant hereto will be in writing and will be deemed to have been duly given on the date delivered, if delivered personally, or on the next Business Day after being sent by reputable overnight courier (with delivery tracking provided, signature required, and delivery prepaid), in each case, to the Parties at the following addresses, or on the date sent (and confirmed by confirmatory return email and by a hard copy delivered by reputable overnight courier (with delivery tracking provided, signature required, and delivery prepaid)) to the email address and address specified below (or at such other address, or email address for a Party as will be specified by notice given in accordance with this Section 16.9 (Notices)).

If to Arrowhead, to:	Arrowhead Pharmaceuticals, Inc. 177 E. Colorado Blvd., Suite 700 Pasadena, CA 91105 Attention: General Counsel Email: [***]
With a copy (which will not constitute notice) to:	Gibson Dunn & Crutcher LLP One Embarcadero Center Suite 2600 San Francisco, CA 94111-3715 Attention: Karen A. Spindler Email: [***]
If to Sarepta, to:	Sarepta Therapeutics, Inc. 215 First Street

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Cambridge, MA 02142
Attention: General Counsel
Email: [***]

With a copy (which will not constitute notice) to:

Ropes & Gray LLP
Prudential Tower
800 Boylston Street
Boston, MA 02199-3600
Attention: Hannah H. England
Email: [***]

16.10. Force Majeure. Each Party will be excused from the performance of its obligations under this Agreement to the extent that such performance is prevented by Force Majeure and such nonperforming Party promptly provides written notice of the prevention to the other Party. The affected Party also will provide a good faith estimate of the period for which its failure or delay in performance under this Agreement is expected to continue based on currently available information and will undertake reasonable efforts necessary to mitigate and overcome such Force Majeure event and resume normal performance of its obligations hereunder as soon a reasonably practicable under the circumstances. If the Force Majeure event continues, the affected Party will update such notice to the other Party on a weekly basis, or more frequently if requested by the other Party, to provide updated summaries of its mitigation efforts and its estimates of when normal performance under the Agreement will be able to resume. Without limiting the affected Party's foregoing obligations, such excuse will be continued so long as the condition constituting Force Majeure continues and such affected Party is exercising reasonable efforts to remedy the Force Majeure. If a Force Majeure persists for more than [***] days, then the Parties will discuss in good faith a modification of the Parties' obligations under this Agreement in order to mitigate the delays caused by such Force Majeure.

16.11. Relationship of the Parties. It is expressly agreed that Arrowhead, on the one hand, and Sarepta, on the other hand, will be independent contractors and that the relationship between the two Parties will not constitute a partnership, joint venture, or agency, including for tax purposes. Neither Arrowhead nor Sarepta will have the authority to make any statements, representations, or commitments of any kind, or to take any action that will be binding on the other, without the prior written consent of the other Party to do so. All Persons employed by a Party will be employees of that Party and not of the other Party and all expenses and obligations incurred by reason of such employment will be for the account and expense of such Party.

16.12. Further Assurances. Each Party will duly execute and deliver, or cause to be duly executed and delivered, such further instruments and do and cause to be done such further acts and things, including the filing of such assignments, agreements, documents, and instruments, as may be necessary or as the other Party may reasonably request, and at such other Party's cost and expense, in connection with this Agreement or to carry out more effectively the provisions and purposes hereof.

16.13. Performance by Affiliates. Each Party may discharge any obligations and exercise any rights hereunder through delegation of its obligations or rights to any of its Affiliates. Each Party hereby guarantees the performance by its Affiliates of such Party's obligations under this Agreement and

will cause its Affiliates to comply with the provisions of this Agreement in connection with such performance.

16.14. Binding Effect; No Third Party Beneficiaries. As of the Effective Date, this Agreement will be binding upon and inure to the benefit of the Parties and their respective permitted successors and permitted assigns. Except as expressly set forth in this Agreement, no Person other than the Parties and their respective Affiliates and permitted assignees hereunder will be deemed an intended beneficiary hereunder or have any right to enforce any obligation of this Agreement.

16.15. Expenses. Except as otherwise provided herein, all fees, costs, and expenses (including any legal, accounting and banking fees) incurred in connection with the preparation, negotiation, execution and delivery of this Agreement and to consummate the transactions contemplated hereby will be paid by the Party hereto incurring such fees, costs, and expenses.

16.16. Counterparts. This Agreement may be executed in two or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument. This Agreement may be executed by facsimile, .pdf, or other electronically transmitted signatures and such signatures will be deemed to bind each Party as if they were the original signatures.

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IN WITNESS WHEREOF, the Parties have caused this Exclusive License and Collaboration Agreement to be executed by their duly authorized representatives as of the Execution Date.

SAREPTA THERAPEUTICS, INC.

BY: /s/ Doug Ingram

NAME: Doug Ingram

TITLE: President and Chief Executive Officer

ARROWHEAD PHARMACEUTICALS, INC.

BY: /s/ Christopher Anzalone, PhD

NAME: Christopher Anzalone, PhD

TITLE: President and Chief Executive Officer

Exhibit 19.1

SAREPTA THERAPEUTICS, INC.

INSIDER TRADING POLICY

1.Purpose. This Insider Trading Policy (this "Policy") provides guidelines with respect to transactions in the securities of Sarepta Therapeutics, Inc. (the "Company") and the handling of confidential information about the Company and the companies with which the Company does business. The Company's Board of Directors (the "Board") has adopted this Policy to promote compliance with U.S. federal and state securities laws that prohibit certain persons who are aware of material nonpublic information about a company from: (i) trading in securities of that company; or (ii) providing such material nonpublic information to other persons who may trade on the basis of that information, commonly known as "tipping."

2.Persons Subject to this Policy. This Policy applies to all directors, officers and employees of the Company and its subsidiaries. This Policy also applies to: (i) family members who reside with them,
(ii) anyone else who lives in their household, (iii) any family members who do not live in their household but whose transactions in Company Securities (as defined below) are directed by them or are subject to their influence or control (such as parents or children who consult with them before they transact in Company Securities) and (iv) family trusts, family partnerships and similar entities controlled by them or any person described in clauses (i)-(iii) (collectively, "Other Covered Persons"). Directors, officers and employees are responsible for transactions by Other Covered Persons and for informing them of this Policy.

The Company also may determine that other persons should be subject to this Policy, such as contractors or consultants who have access to material nonpublic information related to the Company. Any such other persons shall be notified by the Policy Administrator (as defined below).

3.Transactions Subject to this Policy. This Policy applies to transactions in the Company's securities (collectively, "Company Securities"), including the Company's common stock, options to purchase common stock, restricted stock units or any other type of security that the Company may issue, including, but not limited to, preferred stock, convertible debt and warrants, as well as derivative securities that are not issued by the Company, such as exchange-traded put or call options or swaps relating to Company Securities.

4.Individual Responsibility. Persons subject to this Policy have ethical and legal obligations to maintain the confidentiality of information about the Company and to not engage in transactions in Company Securities while in possession of material nonpublic information. Each person is responsible for making sure that he or she complies with this Policy, and that any Other Covered Person also complies with this Policy. In all cases, the responsibility for determining whether a person is in possession of material nonpublic information rests with that person, and any action on the part of the Company, the Policy Administrator or any other employee or director pursuant to this Policy (or otherwise) does not in any way constitute legal advice or insulate a person from liability under applicable securities laws. Persons subject to this Policy could be subject to severe legal penalties and disciplinary action by the Company for any conduct prohibited by this Policy or applicable securities laws, as described below in more detail
under the heading "Consequences of Violations."

5. Administration of this Policy. The Company's General Counsel shall serve as the
compliance officer for the purposes of this Policy (the "Policy Administrator"), and in such role, shall be responsible for the administration of this Policy. In the absence of the Policy Administrator, another employee designated by the Policy Administrator (or, if the Policy Administrator is unavailable, by the Chief Executive Officer) shall be responsible for administration of this Policy. All determinations and interpretations by the Policy Administrator or his or her delegate shall be final and not subject to further

review.

6. Statement of Policy. The policy of the Company is that a director, officer or other employee of the Company or its subsidiaries (or any other person designated by this Policy or by the Policy Administrator or his or her delegate as subject to this Policy) who is aware of material nonpublic information relating to the Company may not directly or indirectly through Other Covered Persons:

- engage in transactions in Company Securities, except as otherwise specified in this Policy under the headings "Transactions Under Company Plans," "Transactions Not Involving a Purchase or Sale" and "Rule 10b5-1 Plans";
- recommend to anyone the purchase or sale of any Company Securities;
- disclose material nonpublic information to persons within the Company whose jobs do not require them to have that information, or anyone outside of the Company, including, but not limited to, family, friends, business associates, investors and expert consulting firms, unless any such disclosure is made in accordance with the Company's policies regarding the protection or authorized external disclosure of information regarding the Company; or
- assist anyone engaged in the above activities in contravention of this Policy.

In addition, the Company's policy is that a director, officer or employee of the Company or its subsidiaries (or any other person designated by this Policy or by the Policy Administrator or his or her delegate as subject to this Policy) who, in the course of working for the Company or its subsides, learns of material nonpublic information about a company with which the Company does business, including a customer or supplier of the Company or any of its subsidiaries, may not transact in that company's securities until the information becomes public or is no longer material.

The only exceptions to this Policy are specified herein. Transactions that may be necessary or justifiable for independent reasons (such as the need to raise money for an emergency expenditure), or small transactions, are not exempt from this Policy. The securities laws do not provide exceptions for mitigating circumstances, and, in any event, even the appearance of an improper transaction must be avoided to preserve the Company's reputation for adhering to high standards of conduct.

Additionally, it is the Company's policy to comply with all applicable securities laws when transacting in its own securities.

7. Definition of Material Nonpublic Information.

7.1. Material Information. Information is considered "material" if a reasonable investor would consider the information important in making a decision to buy, hold or sell securities. Information expected to affect a company's stock price, whether positive or negative, should be considered material. No bright-line standard exists for assessing materiality; rather, materiality is based on an assessment of all of the facts and circumstances and often is evaluated by enforcement authorities with the benefit of hindsight.

While defining all categories of material information is not possible, the following are some examples of information that ordinarily would be regarded as material:

- projections of future earnings or losses, or other financial guidance;
- changes to previously announced financial guidance, or the decision to suspend financial guidance;
- a pending or proposed merger, acquisition or tender offer;

- a pending or proposed acquisition or disposition of a significant asset;
- a pending or proposed significant joint venture or licensing arrangement;
- a Company restructuring;
- significant related party transactions;
- a change in dividend policy, the declaration of a stock split or an offering of additional securities;
- bank borrowings or other financing transactions out of the ordinary course;
- the establishment of a repurchase program for Company Securities;
- a change in the Company's pricing or cost structure;
- a change in management;
- a change in the auditor or notification that the auditor's reports may no longer be relied upon;
- pending or threatened significant litigation, or the resolution of such litigation;
- significant regulatory developments;
- results of clinical trials;
- timelines for expected launches of new products;
- significant changes or developments in supplies or inventory of products, including product defects, recalls or product returns;
- impending bankruptcy or the existence of severe liquidity problems;
- the gain or loss of a significant customer or supplier;
- a significant cybersecurity breach or incident; and
- the imposition of a ban on trading in Company Securities or the securities of another company.

7.2. Nonpublic Information. Generally, information that has not been disclosed to the public is considered to be nonpublic information. In order to establish that the information has been disclosed to the public, it may be necessary to demonstrate that the information has been widely disseminated through major newswire services, national news services, financial news services or public disclosure documents filed or furnished to the Securities and Exchange Commission (the "SEC") that are available on the SEC's website. By contrast, information would generally not be considered widely disseminated if it is available only to the Company's employees.

Once information is widely disseminated, the investing public should be afforded sufficient time to absorb the information. As a general rule, information is considered nonpublic until the end of the first full trading day after the information is released. For example, if the Company announces financial results after market close on Monday or before trading begins on a Tuesday, the first time a director, officer or employee can transact in Company Securities is the opening of the market on Wednesday (assuming he or she is not aware of other material nonpublic information at that time). If the Company announces financial results after trading begins on that

Tuesday, however, the first time a director, officer or employee can transact in Company Securities is the opening of the market on Thursday (again assuming he or she is not aware of other material nonpublic information at that time). Depending on the particular circumstances, the Company may determine that a longer or shorter period should apply to the release of specific material nonpublic information.

8. Transactions Under Company Plans. This Policy does not apply in the case of the following transactions, except as specifically noted:

8.1. Stock Option Exercises. This Policy does not apply to the exercise of a stock option acquired pursuant to a Company equity incentive plan, to the delivery of Company shares to the Company to pay the exercise price of a stock option acquired pursuant to a Company equity incentive plan, or to the exercise of a tax withholding right pursuant to which a person elects to have the Company withhold shares subject to an option award to satisfy tax withholding requirements. This Policy does, however, apply to any sale of shares as part of a broker-assisted cashless exercise of an option, or any other market sale for the purpose of generating the cash needed to pay the exercise price of or taxes associated with an option.

8.2. Restricted Stock and Similar Awards. This Policy does not apply to the vesting of restricted stock, the settlement of restricted stock units or similar awards or to the exercise of a tax withholding right pursuant to which a person elects to have the Company withhold shares to satisfy tax withholding requirements upon the vesting of any restricted stock or the vesting or settlement of any restricted stock unit. This Policy does apply, however, to any market sale of shares received upon the vesting of restricted stock or the settlement of any restricted stock unit or similar award.

8.3. Employee Stock Purchase Plan. This Policy does not apply to periodic purchases under a Company employee stock purchase plan, if such plan exists, that are made as the result of an election made at the beginning of the purchase period. This Policy would apply, however, to an initial decision to participate in the plan or a decision to increase or decrease the level of contribution in a subsequent purchase period. This Policy also applies to any sales of shares purchased under the plan.

8.4. 401(k) Plan. This Policy does not apply to purchases of Company Securities in the Company's or its subsidiaries' 401(k) plans, if any, as a result of periodic contributions made pursuant to payroll deduction. The Policy does apply, however, to initial elections to participate, and increases or decreases in the level of participation, in a Company stock fund and transfers in or out of a Company stock fund (including in connection with a plan loan).

9. Transactions with the Company. Any purchase of Company Securities from the Company or sales of Company Securities to the Company not already identified in Section 8 are not subject to this Policy.

10. Transactions Not Involving a Purchase or Sale. *Bona fide* gifts and transfers to trusts for no consideration are not subject to this Policy, unless (i) the person making the gift or transfer knew or was reckless in not knowing that the recipient would sell the securities while the director, officer, or employee (or Other Covered Person) was aware of material nonpublic information or (ii) the person making the gift or transfer is subject to the trading restrictions specified below under the heading "Trading Window and Pre-Clearance Procedures" and the sale by the recipient of the Company Securities occurs during the current Blackout Period (as defined in the Addendum to this Policy).

In addition, transactions in mutual funds and other broad-based indices or securities that are invested in Company Securities are not transactions subject to this Policy.

11.Special and Prohibited Transactions. The Company has determined that the following transactions present a heightened legal risk and the potential appearance of improper or inappropriate conduct. The Company's policy, consequently, is that the persons identified below must abide by the following restrictions:

11.1.Short-Term Trading. Short-term trading of Company Securities may be distracting to the person engaging in such trades and may unduly focus such person on the Company's short- term performance instead of the Company's long-term objectives. For these reasons, any director or executive officer of the Company who purchases Company Securities may not sell any Company Securities of the same class during the six months following the purchase (or vice versa). In accordance with Section 16(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), any profits received from prohibited short-term trades must be disgorged to the Company.

11.2.Short Sales. Short sales of Company Securities (*i.e.,* the sale of a security that the seller does not own) may reduce a seller's incentive to seek to improve the Company's performance, if the seller could benefit from a decline in value of Company Securities. In addition, short sales may evidence an expectation on the part of the seller that the securities will decline in value and, therefore, have the potential to signal to the market that the seller lacks confidence in the Company's prospects. For these reasons, persons covered by this Policy are prohibited from engaging in any short sales of Company Securities. In addition, Section 16(c) of the Exchange Act prohibits executive officers and directors from engaging in short sales. Short sales arising from certain types of hedging transactions are also governed by the paragraph below captioned "Hedging Transactions."

11.3.Publicly Traded Options. Given the relatively short term of publicly traded options, transactions in options may create the appearance that a director, officer or employee is trading based on material nonpublic information and focus such person's attention on short-term performance at the expense of the Company's long-term objectives. Accordingly, persons covered by this Policy are prohibited from engaging in any transactions in put options, call options or other derivative securities on an exchange or in any other organized market.

11.4.Hedging Transactions. Hedging or monetization transactions can be accomplished through a number of possible mechanisms, including through the use of financial instruments such as prepaid variable forwards, equity swaps, collars and exchange funds. Such transactions may permit a director, officer or employee to continue to own Company Securities obtained through employee benefit plans or otherwise without the full risks and rewards of ownership. When that occurs, such person may no longer have the same objectives as the Company's other shareholders. Therefore, persons covered by this Policy are prohibited from engaging in any such transactions.

11.5.Margin Accounts and Pledged Securities. Securities held in a margin account as collateral for a margin loan may be sold by the broker without the customer's consent if the customer fails to meet a margin call. Similarly, securities pledged (or hypothecated) as collateral for a loan may be sold in foreclosure if the borrower defaults on the loan. Because a margin sale or foreclosure sale may occur at a time when the pledgor is aware of material nonpublic information or otherwise is not permitted to trade in Company Securities, persons covered by this Policy are prohibited from holding Company Securities in a margin account or otherwise pledging Company Securities as collateral for a loan.

11.6.Standing and Limit Orders. Standing and limit orders (except standing and limit orders under Rule 10b5-1 Plans, as described below) create heightened risks for insider trading

violations similar to the use of margin accounts. There is no control over the timing of purchases or sales that result from standing instructions to a broker, and as a result the broker could execute a transaction when a person is in possession of material nonpublic information. The Company therefore discourages placing standing or limit orders on Company Securities other than pursuant to Rule 10b5-1 Plans. If a person subject to this Policy determines that they must use a standing order or limit order, the order should be placed during a window in which the person has received pre-clearance from the Policy Administrator and be limited to a duration of no more than seven days from the date of such pre-clearance. If a standing order or limit order is considered a trading plan under Rule 10b5-1, the person may only enter into such standing order or limit order if permitted under Rule 10b5-1, including, giving effect to any limitations on overlapping plans under Rule 10b5-1.

12. Rule 10b5-l Plans. Rule 10b5-1 under the Exchange Act provides a defense from insider trading liability under Rule 10b-5. In order to be eligible to rely on this defense, a person subject to this Policy must enter into a contract, instruction or written plan for transactions in Company Securities that meets certain conditions specified in the rule (a "Rule 10b5-l Plan"). If the plan meets the requirements of Rule 10b5-1, Company Securities may be purchased or sold without regard to certain insider trading restrictions. To comply with this Policy, a Rule 10b5-1 Plan must be approved by the Policy Administrator and meet the requirements of Rule 10b5-l. A Rule 10b5-1 Plan must be entered into in good faith and at a time when the person entering into the plan is not aware of material nonpublic information and may not be adopted during the quarterly Blackout Periods.

Rule 10b5-l Plans shall be considered by the Policy Administrator on a case-by-case basis. Any Rule 10b5-1 Plan shall be submitted to the Policy Administrator for approval at a reasonable time prior to the entry into the Rule 10b5-1 Plan. Multiple overlapping plans will not be approved unless they are exclusively replacement plans, that is, adopted to continue an existing trading plan with no trades executed prior to the expiration of the current plan, or exclusively for the purpose of selling shares to cover tax obligations associated with equity award vesting. Any modification or change to the amount, price or timing of a trade under a Rule 10b5-1 Plan will be treated as a termination of the existing plan and the adoption of a new plan on the modified terms. Once the relevant waiting period has passed, no further pre- approval of transactions conducted pursuant to the Rule 10b5-1 Plan shall be required.

Each quarter, the Company is required to publicly disclose when directors or officers adopt, terminate or make certain modifications to Rule 10b5-1 Plans and other trading plans for Company Securities and to provide a description of the material terms of each plan. Therefore, directors and officers must provide the Policy Administrator with (i) the final executed copy of any 10b5-1 Plan, (ii) any other trading plan for Company Securities and (iii) any amendment to any such Rule 10b5-1 Plan or other trading plan, in each case within two business days of the adoption thereof. In addition, directors and officers must promptly notify the Policy Administrator of any termination or modification of such Rule 10b5-1 Plans or other trading plans.

13.Trading Window and Pre-Clearance Procedures. To help prevent inadvertent violations of the federal securities laws and to avoid even the appearance of trading on the basis of inside information, the Board has adopted an Addendum to this Policy that applies to those persons who have regular access to material nonpublic information about the Company and are identified in the Addendum. The Company shall notify those persons who are subject to the Addendum.

The Addendum generally prohibits persons and entities covered by it from trading in Company Securities, except during the trading windows specified in the Addendum and requires that certain persons shall pre-clear all transactions in Company Securities with the Policy Administrator.

In addition, from time to time, the Company may be involved in activities—such as proposed acquisitions—that are material and that are known only by a few people at the Company. For those persons whose duties at the Company cause them to be aware of such activity, the Policy Administrator shall notify them of an event-specific trading restriction, and those persons shall not be permitted to trade in Company Securities during such trading restriction. The existence of an event-specific trading restriction shall not be widely announced and should not be communicated to anyone. Even if persons are not notified of an event-specific trading restriction, they should not trade in Company Securities if they are aware of material nonpublic information.

14.Post-Termination Transactions. This Policy continues to apply to transactions in Company Securities even after termination of service to the Company or its subsidiaries. If a person is in possession of material nonpublic information when his or her service terminates, that person may not trade in Company Securities until that information has become public or is no longer material.

15.Consequences of Violations. The purchase or sale of securities while aware of material nonpublic information, or the disclosure of material nonpublic information to others who then trade in the Company's Securities, is prohibited by U.S. federal and state laws. Insider trading violations are pursued vigorously by the SEC, U.S. Attorneys and state enforcement authorities as well as foreign regulatory authorities. Punishment for insider trading violations is severe, and could include significant fines and imprisonment. While the regulatory authorities concentrate their efforts on the individuals who trade, or who tip inside information to others who trade, the federal securities laws also impose potential liability on companies and other "controlling persons" if they fail to take reasonable steps to prevent insider trading by company personnel.

In addition, a person's failure to comply with this Policy may subject such person to Company- imposed sanctions, including dismissal for cause, whether or not the person's failure to comply results in a violation of law. In addition to the formal sanctions summarized above, a violation of law, or even an SEC investigation that does not result in prosecution, can tarnish a person's reputation and irreparably damage a career.

16.Company Assistance. Any person who has a question about this Policy or its application to any proposed transaction may obtain additional guidance from the Policy Administrator, who can be reached by telephone at 617-301-8692 or by email at crothfuss@sarepta.com.

Effective: December 8, 2023

ADDENDUM TO INSIDER TRADING POLICY

The Company has established additional procedures to assist in the administration of the Insider Trading Policy, to facilitate compliance with laws prohibiting insider trading while in possession of material nonpublic information and to avoid the appearance of any impropriety. These additional procedures are applicable only to the persons specified below and other persons who are periodically designated by the Policy Administrator or his or her delegate as being subject to these additional procedures (as well as their family members, household members and entities whose transactions are subject to the Insider Trading Policy). The Policy Administrator shall notify those persons who are subject to this Addendum. All capitalized terms used herein and not otherwise defined shall have the respective meanings set forth in the Policy.

In all cases, the responsibility for determining whether a person is in possession of material nonpublic information rests with that person, and any action on the part of the Company, the Policy Administrator or any other employee or director pursuant to the Insider Trading Policy, including this Addendum (or otherwise), does not in any way constitute legal advice or insulate a person from liability under applicable securities laws.

1. Pre-Clearance Procedures. The Company's directors, the Company's Chief Executive Officer, Executive Vice Presidents, Senior Vice Presidents, Vice Presidents and other persons who are designated by the Policy Administrator or his or her delegate as being subject to this Addendum (as well as their family members, household members and entities whose transactions are subject to the Insider Trading Policy) (collectively, the "Covered Persons") may not engage in any transaction in Company Securities at any time (other than as specified by the Insider Trading Policy, including this Addendum), even if not subject to a Blackout Period, without first obtaining pre-clearance of the transaction from the Policy Administrator or his or her delegate. The General Counsel may not engage in any transaction in Company Securities at any time (other than as specified by the Insider Trading Policy, including this Addendum), even if not subject to a Blackout Period, without first obtaining pre-clearance of the transaction from the Chief Executive Officer. The Policy Administrator is under no obligation to approve a transaction submitted for pre-clearance, and may determine not to permit the transaction for any or no reason. If a Covered Person seeks pre-clearance and permission to engage in the transaction is denied, then he or she should refrain from initiating any transaction in Company Securities, and should not inform any other person of the restriction.

When a request for pre-clearance is made, the requestor should carefully consider whether he or she may be aware of any material nonpublic information about the Company and should describe fully those circumstances to the Policy Administrator. If subject to Section 16 of the Exchange Act, the requestor also should indicate whether he or she has effected any nonexempt "opposite-way" transactions within the past six months, and should be prepared to report the proposed transaction on an appropriate Form 4 or Form 5, if applicable. The requestor should also be prepared to comply with Rule 144 under the Securities Act of 1933, as amended, and file a Form 144, if necessary, at the time of any sale. After receiving clearance to engage in a transaction from the Policy Administrator, the requestor shall complete the proposed transaction within seven days or make a new transaction request.

2. Quarterly Trading Restrictions. The Company's directors, Executive Committee members, and other persons who are designated by the Policy Administrator or his or her delegate as being subject to this Addendum, including certain members of the Company's finance, commercial, legal and investor relation teams (as well as their family members, household members and entities whose transactions are subject to the Insider Trading Policy) may not engage in any transaction in Company Securities (other than as specified by the Insider Trading Policy,

including this Addendum) during a "Blackout Period" beginning two weeks prior to the end of each fiscal quarter and ending after the first full trading day following the date of the public release of the Company's earnings results for that quarter. In other words, these persons may only conduct transactions in Company Securities during the "Window Period" beginning on the day after the first full trading day following the public release of the Company's quarterly earnings and ending two weeks prior to the close of the next fiscal quarter.

3. Exceptions. The quarterly trading restrictions described above do not apply to those transactions to which the Insider Trading Policy does not apply, as described in the Insider Trading Policy under the headings "Transactions Under Company Plans," "Transactions Not Involving a Purchase or Sale" and "Transactions with the Company." Further, the requirement for pre-clearance, the quarterly trading restrictions and event-specific trading restrictions do not apply to transactions conducted pursuant to approved Rule 10b5-l Plans described in the Insider Trading Policy under the heading "Rule 10b5-l Plans."

Effective: December 8, 2023

EXHIBIT 21.1

Sarepta Therapeutics, Inc.
Subsidiaries of the Registrant

Name	Jurisdiction of Incorporation
Sarepta Securities Corp.	Massachusetts, USA
ST International Holdings, Inc.	Delaware, USA
ST International Holdings Two, Inc.	Delaware, USA
Sarepta Therapeutics Two, LLC	Delaware, USA
Sarepta Therapeutics Three, LLC	Delaware, USA
Sarepta Therapeutics Ireland LLP	Ireland
Sarepta Therapeutics Ireland Two LLP	Ireland

EXHIBIT 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the registration statements (Nos. 333-263208 and 333-234698) on Form S-3ASR and (Nos. 333-273608, 333-266461, 333-240996, 333-233715, 333-228719, 333-221271, 333-213022, 333-209710, 333-199037, 333-192287, 333-175031, 333-172823, 333-101826, 333-49996, 335-49994 and 333-34047) on Form S-8 of our report dated February 28, 2025, with respect to the consolidated financial statements of Sarepta Therapeutics, Inc. and the effectiveness of internal control over financial reporting.

/s/ KPMG LLP

Boston, Massachusetts
February 28, 2025

EXHIBIT 31.1

CERTIFICATION

I, Douglas S. Ingram, certify that:

1. I have reviewed this Annual Report on Form 10-K of Sarepta Therapeutics, Inc., (the "Registrant");

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the Registrant as of, and for, the periods presented in this report;

4. The Registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the Registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the Registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the Registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the Registrant's internal control over financial reporting that occurred during the Registrant's most recent fiscal quarter (the Registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the Registrant's internal control over financial reporting; and

5. The Registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Registrant's auditors and the audit committee of the Registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the Registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the Registrant's internal control over financial reporting.

February 28, 2025

/s/ Douglas S. Ingram

Douglas S. Ingram
President and Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

CERTIFICATION

I, Ian M. Estepan, certify that:

1. I have reviewed this Annual Report on Form 10-K of Sarepta Therapeutics, Inc., (the "Registrant");

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the Registrant as of, and for, the periods presented in this report;

4. The Registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the Registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the Registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the Registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the Registrant's internal control over financial reporting that occurred during the Registrant's most recent fiscal quarter (the Registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the Registrant's internal control over financial reporting; and

5. The Registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Registrant's auditors and the audit committee of the Registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the Registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the Registrant's internal control over financial reporting.

February 28, 2025

/s/ Ian M. Estepan

Ian M. Estepan

Executive Vice President, Chief Financial Officer

(Principal Financial and Accounting Officer)

EXHIBIT 32.1

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Douglas S. Ingram, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that the Annual Report of Sarepta Therapeutics, Inc. on Form 10-K for the fiscal year ended December 31, 2024, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Annual Report on Form 10-K fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

February 28, 2025

/s/ Douglas S. Ingram
Douglas S. Ingram
President and Chief Executive Officer
(Principal Executive Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Report on Form 10-K pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.

EXHIBIT 32.2

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Ian M. Estepan, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that the Annual Report of Sarepta Therapeutics, Inc. on Form 10-K for the fiscal year ended December 31, 2024, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Annual Report on Form 10-K fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

February 28, 2025

/s/ Ian M. Estepan
Ian M. Estepan
Executive Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Report on Form 10-K pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.