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, 2016

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:

Tile of Each Class	**Name of Exchange on Which Registered**
Common Stock, $0.0001 par value	**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 Section 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 and posted on its corporate Website, if any, every Interactive Data File required to be submitted and posted 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 and post such files). Yes ☒ No ☐

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) is not contained herein, and will not be contained, to the best of the registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☒

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

Large accelerated filer	☒	Accelerated filer	☐
Non-accelerated filer	☐ (Do not check if a smaller reporting company)	Smaller reporting company	☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). Yes ☐ No ☒

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of June 30, 2016 was approximately $912,884,809.

The number of outstanding shares of the registrant's common stock as of the close of business on February 21, 2017 was 54,813,260.

DOCUMENTS INCORPORATED BY REFERENCE

The registrant has incorporated by reference into Part III of this Annual Report on Form 10-K, portions of its definitive Proxy Statement for its 2017 annual meeting to be filed with the Commission no later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K.

Sarepta Therapeutics, Inc.
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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. The statements contained in this Annual Report on Form 10-K that are not purely historical are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. 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. You should read these statements carefully because they discuss future expectations, contain projections of future results of operations or financial condition, or state 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 ability to achieve a successful commercial launch of EXONDYS 51 in the U.S., including through executing our plans to hire additional personnel, increase awareness on the importance of genetic testing and knowing / understanding DMD mutations, and identify and address procedural barriers for patients to obtain therapy such as payor reimbursement challenges, maintaining the marketing, distribution and supply infrastructure we have built for EXONDYS 51 and our expectations regarding the timing, costs, and investments associated with these activities;*

- *our ability to obtain full approval of eteplirsen (i) in the U.S., which is dependent on our ability to complete to the United States Food and Drug Administration's ("FDA") satisfaction our post-marketing requirements and commitments, (ii) in the EU, which is dependent on our ability to successfully navigate the EU drug approval process and (iii) in other parts of the world that we may target;*

- *the potential acceptance of EXONDYS 51, and our product candidates if they receive regulatory approval, in the marketplace and the accuracy of our projections regarding the market size in each of the jurisdictions that we target;*

- *our ability to further secure long term supply of EXONDYS 51 and our product candidates, including by securing supply of subunits, drug substance Active Pharmaceutical Ingredients ("APIs") and drug product, to satisfy our planned commercial and clinical needs by, among other things, negotiating and entering into additional commercial and supply agreements, and further scaling up manufacturing using appropriate techniques to synthesize and purify our product candidates to meet regulatory, Company quality control and other applicable requirements;*

- *our expectations regarding our ability to successfully conduct or accelerate research, development, pre-clinical, clinical and post-approval trials, and our expectations regarding the timing, design and results of such trials, including their potential consistency with prior results, as well as data and analyses relating to the safety profile and potential clinical benefits of EXONDYS 51 and our product candidates;*

- *our success in advancing the development of our follow-on exon-skipping drug candidates targeting DMD and further exploring potential funding, collaborations and other opportunities to support such development;*

- *the potential and advancement of our phosphorodiamidate morpholino oligomer ("PMO") chemistries, our peptide-conjugated PMO ("PPMO") chemistries, our other PMO-based chemistries, and our other technologies to treat Duchenne muscular dystrophy ("DMD") and other diseases and therapeutic areas that we target;*

- *our ability to successfully expand the global footprint of eteplirsen, including through obtaining an approval from the European Medicines Agency ("EMA") in the EU and other jurisdictions where regulatory approval has not been obtained, establishing early access programs in other parts of the world, building the internal infrastructure for commercialization and ensuring commercial supply to support these plans, and our expectations regarding the EMA review process, including timing and the factors that will impact the EMA's evaluation and decision;*

- *the impact of regulations and regulatory decisions by 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 any competing products on the commercial success of EXONDYS 51 and our product candidates and our ability to compete against such products;*

- *the impact of potential difficulties in product development manufacturing, or the commercialization of EXONDYS 51 and our product candidates, including factors such as successfully establishing and maintaining the appropriate Company infrastructure necessary to support the Company's research, development and commercialization efforts;*

- *our expectations regarding our ability to become a leading developer and marketer of PMO-based and RNA-targeted therapeutics and commercial viability of EXONDYS 51, as well as our product candidates, chemistries and technologies;*

- *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 extent of protection that our patents provide and our pending patent applications may provide, if patents issue from such applications, to our technologies and programs, and our ability to maintain patent protection for our technologies and programs;*

- *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 ability to invalidate some or all of the claims of patents issued to competitors and pending patent applications if issued to competitors, and the potential impact of those claims on the potential commercialization and continued commercialization, where authorized, of EXONDYS 51 and the potential commercialization of our product candidates;*

- *our ability to successfully challenge the patent positions of our competitors and successfully defend our patent positions in the actions that the United States Patent and Trademark Office (the "USPTO") or any appeals court may take or has taken with respect to our patent claims or those of third parties, including any appeals in connection with the interference decisions regarding our patents and patent applications and those held by BioMarin Pharmaceutical, Inc., ("BioMarin") relating to exon 51, including EXONDYS 51, and exon 53, including SRP-4053, and our expectations regarding the impact of any appeal decisions in connection with these interferences on our business plans, including our commercialization for EXONDYS 51 and, if authorized, SRP-4053;*

- *the impact of any consequences of the interference decisions and ongoing appeals including the final refusal of BioMarin claims in the exon 53 and exon 51 composition of matter interferences, the cancellation of our patent in the exon 51 method of use interference, and the narrow claim BioMarin was allowed to pursue as a result of the exon 53 interference decision;*

- *the impact if the USPTO, other agencies or courts make a decision against us that could negatively impact the EXONDYS 51 commercialization such as a decision in the pending appeals of Interference Nos. 106,007, 106,008 and 106,013, any of which could result in an infringement claim against us if the BioMarin patent applications subject to the appeals are ultimately granted;*

- *our ability to operate our business without infringing the intellectual property rights of others;*

- *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 ability to raise additional funds to support our business plans and strategies, including business development, and the impact of our credit and security agreement with MidCap Financial on our financial condition and future operations;*

- *our expectations relating to potential funding from government and other sources for the development of some of our product candidates;*

- *the timing and outcomes of ongoing interference proceedings and related appeals, and the impact of any litigation on us, including actions brought by stockholders;*
- *our ability to attract and retain key employees needed to execute our business plans and strategies and our expectations regarding our ability to manage the impact of any loss of key employees;*
- *our ability to comply with applicable environmental laws and regulations;*
- *the impact of the potential achievement of performance conditions and milestones relating to our stock awards; 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 ("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.

PART I

Item 1. Business.

Overview

We are a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. Applying our proprietary, highly-differentiated and innovative platform technologies, we are able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action.

DMD Focus

We are primarily focused on rapidly advancing the development of our potentially disease-modifying pipeline of exon-skipping drug candidates targeting DMD. DMD is a rare genetic disorder affecting children (primarily males) that characterized by progressive muscle deterioration and weakness. It is the most common type of muscular dystrophy. DMD is caused by an absence of dystrophin, a protein that helps keep muscle cells intact. The absence of dystrophin in muscle cells leads to significant cell damage and ultimately causes muscle cell death and fibrotic replacement. Females are rarely affected by the disorder. 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 before the age of 30, to which they typically succumb.

EXONDYS 51 is the first disease-modifying therapy to receive accelerated approval for DMD in the U.S. The yearly cost of care for individuals with DMD is high and increases with disease progression. We believe that DMD represents a significant market opportunity.

U.S. Commercialization

Our first commercial product in the U.S., EXONDYS 51® (eteplirsen) Injection ("EXONDYS 51"), was granted accelerated approval by the FDA on September 19, 2016. EXONDYS 51 is indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. The accelerated approval of EXONDYS 51 is based on the surrogate endpoint of an increase of dystrophin in skeletal muscle observed in some EXONDYS 51-treated patients. The FDA has concluded that the data we submitted demonstrated an increase in dystrophin production that is reasonably likely to predict clinical benefit in some patients with DMD who have a confirmed mutation of the dystrophin gene amenable to exon 51 skipping. A clinical benefit of EXONDYS 51, including improved motor function, has not been established. Continued approval for this indication is contingent upon completing various post-marketing requirements and commitments to confirm the clinical benefit of EXONDYS 51. Our FDA post marketing requirements and commitments include several studies for eteplirsen and SRP-4045 and SRP-4053, our product candidates designed to skip exons 45 and 53, respectively.

In addition to successfully completing our FDA post-marketing requirements and commitments, we are focused on executing our various strategic initiatives designed to drive commercial success of EXONDYS 51 in the U.S. We believe that the key priorities for a successful commercial launch include:

- working with physicians to identify patients and to initiate the process of securing prescriptions by providing education on the importance of genetic testing and knowing / understanding DMD mutations;
- maintaining an active dialogue and working with payors to provide broad reimbursement and coverage for EXONDYS 51;
- identifying and appropriately addressing procedural barriers patients face in obtaining therapy to shorten timeframe to treatment; and
- ensuring all DMD patients have genetic tests and are appropriately identified for exon amenability.

Our commercial initiatives are designed to support these priorities. We are pursuing broad coverage of EXONDYS 51. The payor environment can be very challenging in the orphan drug space. As of the date of this report, the majority of covered lives are pending policy decisions and are currently reviewed case-by-case or approved with restrictions. We continue to have discussions with payors who have denied coverage or have policy decisions limiting coverage of EXONDYS 51.

Ex-U.S. Efforts

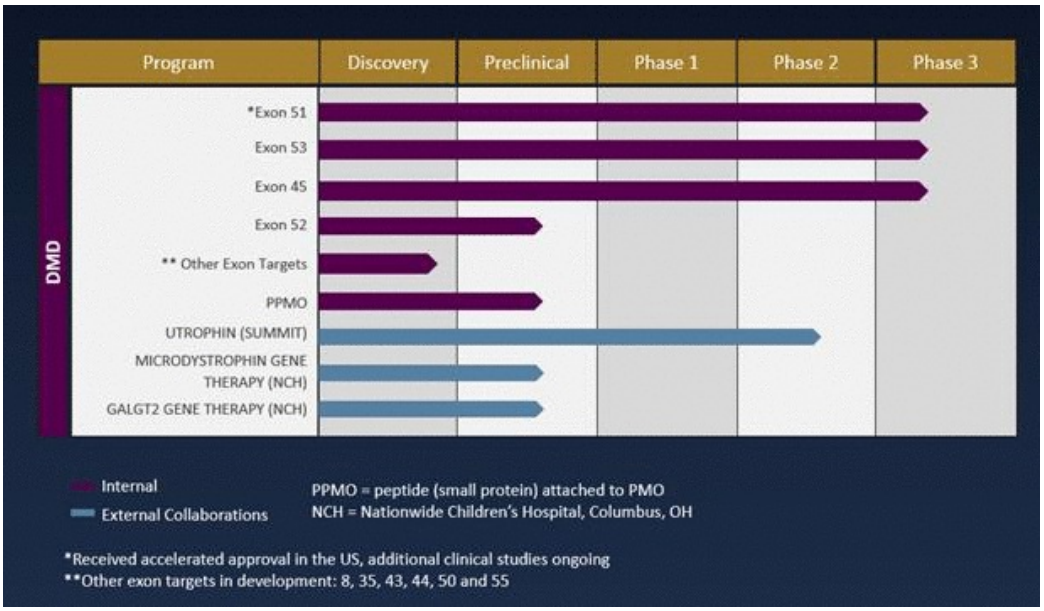
We are also pursuing regulatory approval of eteplirsen in jurisdictions outside of the U.S. On December 19, 2016, we announced that the EMA validated our previously submitted Marketing Authorization Application ("MAA") for eteplirsen to treat DMD amenable to exon 51 skipping. We are seeking conditional approval of eteplirsen in the EU through the centralized procedure. The review of an MAA generally takes approximately 12-15 months. The review time can vary as it is dependent on the "clock stop" period which is generally between 3 to 6 months to provide responses to questions from the EMA throughout the review process. During the review, we plan to initiate key activities in support of the potential launch of eteplirsen in the EU, such as building out our commercial infrastructure and scaling-up manufacturing. We believe Europe presents a large opportunity for us since there are more DMD patients in Europe than in the U.S. As of the date of this report, EXONDYS 51 has not been approved for sale or marketing by any regulatory agency or authority outside of the U.S. We are also working towards establishing early access programs for eteplirsen in various ex-U.S. jurisdictions. For commercialization activities in jurisdictions outside of the U.S., we may choose to conduct these activities ourselves or may enter into arrangements with other pharmaceutical or biotechnology companies for the marketing and sale of our products either globally or on a country-by-country basis.

Our Next Generation Exon Skipping Technology

EXONDYS 51 is our first PMO-based technology to be granted accelerated approval by the FDA. The reminder of our product candidates currently in the clinic are also PMO-based. We are currently focused on rapidly advancing our next generation chemistry for DMD and other applications – a cell penetrating peptide conjugated PMO referred to as PPMO. In pre-clinical research, our proprietary class of PPMO compounds demonstrated enhanced efficacy as compared to PMO by improving delivery and dystrophin production in vivo, and showing a favorable tolerability in non-human primates. Based on these results, we believe our PPMO chemistry has the potential for a more durable response, which may support less frequent dosing and can potentially be tailored to target any organ. Our current plans are to open file an investigation new drug ("IND") application in 2017 for PPMO toxicology studies and DMD exon 51.

Pipeline: A Comprehensive Approach to DMD

In addition to EXONDYS 51, our DMD pipeline includes other product candidates, which are at various stages of development. The chart below summarizes the status of our more advanced programs as of the date of this report, including those with our strategic partners, reflecting our efforts to become the global leader in DMD RNA-targeted precision medicine while also developing a pipeline with a comprehensive treatment approach to DMD:



Collaborations with Multiple Treatment Approaches to DMD

In addition to advancing our exon-skipping product candidates for DMD, we are working with several strategic partners under various agreements to research and develop multiple treatment approaches to DMD. Included in these strategic partners are (i) Summit (Oxford) Ltd. ("Summit") with whom we are collaborating under an exclusive License and Collaboration Agreement that grants us rights to Summit's utrophin modulator pipeline in Europe, Turkey and the Commonwealth of Independent States and an option to acquire rights in Latin America and (ii) Nationwide Children's Hospital with whom we are collaborating on the advancement of their microdystrophin gene therapy program under a research and exclusive option agreement and their Galgt2 gene therapy program under an exclusive license agreement.

Objectives and Business Strategy

We believe that our proprietary RNA-targeted technologies platform 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 RNA-targeted technologies platform, organizational capabilities and resources to become a leading developer and marketer of RNA-targeted therapeutics, including the treatment of rare, neuromuscular and other diseases, with a diversified portfolio of product candidates. In pursuit of this objective, we intend to engage in the following activities:

- executing on our strategic initiatives designed to achieve a successful commercialization of EXONDYS 51 in the U.S.;
- expanding the global footprint of eteplirsen by pursuing regulatory approval or establishing early access programs in jurisdictions where regulatory approval has not yet been obtained;
- advancing the development of our follow-on exon-skipping drug candidates targeting DMD and further exploring potential funding, collaborations and other opportunities to support such development;
- advancing the research and development of our RNA-targeted technologies platform, including our next generation chemistry, PPMO, and identifying product candidates to target additional therapeutic areas at our company or through potential strategic opportunities including potentially entering into partnership, licensing and/or collaboration arrangements with industry partners;
- continuing to expand our portfolio therapeutic approaches targeting DMD through strategic partnerships, collaborations and other potential opportunities; and
- ensuring we have the appropriate capitalization to fund our business objectives and strategies, including by raising additional capital through licensing, collaborations and offerings of Company equity and / or debt.

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. On July 12, 2012, our common stock began trading under the symbol "SRPT" on the NASDAQ Global Market on a split-adjusted basis following a one-for-six reverse stock split that was effective on July 11, 2012. Our common stock is quoted on the NASDAQ Global Select Market under the same symbol.

We have not generated significant revenue from product sales to date. Even if we do achieve significant revenue from product sales, we are likely to continue to incur operating losses in the near term. 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, 2016, we had approximately $329.3 million of cash, cash equivalents and investments, consisting of $122.4 million of cash and cash equivalents, $195.4 million of short-term investments and $11.5 million of restricted cash and investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve 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 public may read and copy any materials we file or furnish with the SEC, at the SEC's Public Reference Room at 100 F Street, N.E., Washington, D.C. 20549 or may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. Moreover, 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 the 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.

Lead Development Program: Pipeline of Exon-Skipping PMO-Based Product Candidates for DMD

Proprietary Platform Technologies

PMO. The original PMO structure and variations of this structure that are so-called PMO-based (collectively "PMO-based") are central to our proprietary chemistry platform. PMO-based therapeutics have been safely dosed in over 400 patients. PMO-based compounds are synthetic compounds that bind to complementary sequences of RNA by standard Watson-Crick nucleobase pairing. When targeted to messenger RNA ("mRNA"), PMO-based compounds down-regulate protein translation by steric blockade. 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 of siRNAs and DNA gapmers, and 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 these other RNA-targeted technologies.

PMO technologies can be used to selectively up-regulate or down-regulate the production of a target protein through pre-mRNA splice alteration. This mechanism can be used to correct disease-causing genetic errors by inducing the targeted expression of

novel proteins. Thus, PMO-based compounds have the potential to be designed to create more, less, or none of certain proteins, or produce analogues of endogenous proteins.

The safety of therapeutic agents is paramount. We believe that our PMO-based compounds significantly reduce the potential for off-target effects specifically because of their demonstrated inactivity with key molecular mechanisms that are known to be toxicologically active when stimulated. Additionally, consistent with our research and development to date, we believe that PMO-based compounds do not exhibit coagulation and immune stimulatory effects, do not stimulate toll-like receptors ("TLRs") or receptors of the RIG-I-like receptor family, and do not sequester metal ions away from the catalytic centers of polymerases.

In addition to our original PMO technology, we have also developed three new PMO-based chemistry platforms. We believe that the novel characteristics intrinsic to these new platforms will allow for the development of drug candidates with favorable safety and efficacy characteristics.

PPMO. The first of these novel chemistries is based on cell-penetrating PPMOs. This technology features covalent attachment of a cell-penetrating peptide to a PMO with the goal of enhanced cellular delivery into the cytosol and the nucleus. Based on our pre-clinical research, we believe our proprietary class of PPMO compounds demonstrates enhanced efficacy as compared to PMO and has the potential to provide improved delivery and dystrophin production in vivo, favorable tolerability in non-human primates, and a more durable response, which may support less frequent dosing and can potentially be tailored to target any organ. We plan on beginning IND-enabling Good Laboratory Practice ("GLP") toxicology studies during early 2017 and we are targeting opening an IND before year-end in 2017 for DMD exon 51.

PMOplus®. The second of these chemistries, PMO*plus*®, features the selective introduction of positive charges to the PMO backbone. We believe that PMO*plus*® has potentially broad therapeutic applications, especially for anti-viral therapeutics.

PMO-X®. The third of these chemistries, PMO-X®, incorporates novel and proprietary chemical modifications to the PMO backbone linkages. We believe PMO-X® may provide enhanced *in vivo* potency and efficacy, as well as greater flexibility in the modulation of selective tissue targeting and cellular delivery.

We believe that our PMO-based technology platforms 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 PMO-based technology platforms, organizational capabilities, and resources to become a leading developer and marketer of a diversified portfolio of PMO-based therapeutics, especially for the treatment of rare, neuromuscular and other diseases.

Exon-Skipping Clinical Studies

The table below indicates the status of our clinical trials for our exon-skipping pipeline as of the date of this filing. This table is a summary description of our research and clinical efforts for each exon skip targeted by our product candidates in our DMD exon-skipping pipeline:

Exon Target Treatment	Study	Duration (weeks)	U.S./EU	Number of Patients	Status	DMD Population
Exon 51	AVI-4658-33	Single Dose	EU	7	Completed	10-17 yrs, non-amb(b)
Exon 51	AVI-4658-28	12	EU	19	Completed	5-15 yrs, amb
Exon 51	4658-us-201	28	US	12	Completed	7-13 yrs, amb
Exon 51	4658-us-202 (a)	268	US	12	Dosing/Enrollment closed (Data through 236 weeks)	7-13 yrs, amb
Exon 51	4658-301	96	US	160	Enrolling	7-16 yrs, amb
Exon 51	4658-204	96	US	24	Dosing/Enrollment closed	7-21 yrs, non-amb
Exon 51	4658-203	96	US	40	Enrolling	4-6 yrs, amb
Exon 51	4658-102	48	EU/US	12	Planned	6 mos - 4 yrs
Exon 45	4045-101	120	US	12	Dosing/Enrollment closed	7-21 yrs, non-amb
Exon 53	4053-101	60	EU	48	Enrollment closed	6-15 yrs, amb
Exon 45/53	4045-301	192	EU/US	99	Enrolling	7-13 yrs, amb

(a) Weeks presented are inclusive of 28 completed weeks in study 4658-us-201.
(b) Amb denotes ambulatory

EXONDYS 51. EXONDYS 51® (eteplirsen) Injection, our first commercial product, approved by the U.S. FDA on September 19, 2016, is indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 targets the most frequent series of mutations that cause DMD. Approximately 13% of DMD patients are amenable to exon 51 skipping. In connection with fulfilling our FDA post-marketing requirements and commitments, we are in the process of planning several studies for eteplirsen and product candidates targeting skipping of exon 53 and/or exon 45. In addition, we are conducting an open label extension of our Phase 2b study for which patients can transition to commercial drug after certain criteria are met (Study 4658-us-202), an open label study on ambulatory patients with a concurrent untreated control arm (Study 4658-301/PROMOVI), a study evaluating the safety and tolerability of eteplirsen in participants with advanced stage DMD (Study 4658-204) and a study evaluating the safety and tolerability of eteplirsen in participants with early stage DMD (Study 4658-203), each of which will allow for patients to transition to commercial drug after meeting certain criteria. We are also planning two additional Phase 1 studies. In connection with our MAA for eteplirsen, which received EMA validation, and our efforts to obtain approval of eteplirsen in the EU, we are in the planning stages of an intravenous study on participants between the ages of six months and four years in connection with our Pediatric Investigation Plan ("PIP") in the EU.

Exon 53. We are enrolling and dosing patients in ESSENCE (Study 4045-301), our phase 3 placebo controlled confirmatory trial in patients who have a confirmed mutation of the DMD gene that is amenable to exon 45 or 53 skipping using SRP-4045 and SRP-4053, respectively. SRP-4053, an exon 53-skipping product candidate that we selected for development through the SKIP-NMD consortium, is currently in the clinic in EU as part of a Phase 1/2 study. Part I has been completed and Part II, an open label portion of this study, is ongoing (Study 4053-101). The SKIP-NMD Consortium, which supported certain clinical proof of concept studies and IND-enabling activities for an exon 53-skipping therapeutic using our PMO technology, received an EU Health Innovation-1 2012 collaborative research grant (grant agreement No. 305370) to support the initial development of SRP-4053. SRP-4053 will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping.

Exon 45. We are enrolling and dosing patients amenable to exon 45 skipping in ESSENCE, our placebo controlled study for eteplirsen further described above. SRP-4045, an exon 45-skipping product candidate that we selected for development in collaboration with Children's National Medical Center ("CNMC") in Washington, D.C. and the Carolinas Medical Center ("CMC") in Charlotte, N.C. This collaboration was funded primarily through two grants, one from Department of Defense's ("DoD") Congressionally Directed Medical Research Program to CNMC and the other from the National Institutes of Neurological Disorders and Stroke to the CMC. Pursuant to an ongoing Sarepta-sponsored Phase 1/2 clinical study studying SRP-4045 (Study 4045-101), we have completed a dose titration portion (Phase 1) and are currently conducting the open-label portion of the study (Phase 2). SRP-4045 will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping.

Our DMD pipeline includes additional product candidates, which are at various stages of development. Our DMD program is part of our larger pan-exon strategy for the development of drug candidates to address the most prevalent exon mutations in the DMD population. Because the majority of DMD patients have exon mutations that cluster together, a small number of exon-skipping therapies will potentially be disease-modifying for a relatively large percentage of DMD patients. Approximately 75-80% of the total DMD population is potentially treatable with exon-skipping therapeutics.

Discovery and Research Programs

Rare Diseases. We are researching the application of our proprietary PMO-based technologies in a number of rare diseases.

Anti-Bacterials. The rapid emergence of broad antibiotic resistance has underscored the urgent need for new paradigms in antimicrobial development. Our anti-bacterial program is focused on drug-resistant bacteria identified by the Centers for Disease Control and Prevention ("CDC") as urgent or serious threats to the U.S. healthcare system. Early research findings demonstrate that targeted PPMOs can successfully inhibit translation of essential structural genes such as acyl carrier protein ("acpP"), resistance proteins such as the NDM-1 metallo-b-lactamase, or those responsible for biofilm formation, which is critical for Burkholderia cepacia complex to evade host immune responses or systemic antibiotics such as cysteine protease cepI., which is responsible for biofilm expression. Additionally, though acpP alone can be bactericidal at clinically achievable concentration, data demonstrates that co-administration of the PPMOs targeting NDM-1 can restore antibiotic activity of drugs like meropenem or imipenem to clinically achievable levels in high-level multidrug resistant Acinetobacter, E. coli, Klebsiella, and Burkholderia spp in both bench top and mouse models. Finally, we have also seen that PPMOs targeting structural genes such as acpP or quorum sensing genes such as cepI (responsible for biofilm expression) can penetrate and disrupt established biofilm; furthermore, the PPMOs targeting acpP can successfully kill the established bacterial colonies in Burkholderia cepacia models. We believe the results of this early research could have broad commercial applicability. We are exploring IND enabling studies now, and are open to partnership opportunities in the development of our anti-bacterial program.

Manufacturing, Supply and Distribution

We have developed proprietary state-of-the-art manufacturing and techniques that allow synthesis and purification of our product and product candidates to support clinical development as well as commercialization. We have entered into certain manufacturing and supply arrangements with third-party suppliers which will in part utilize these techniques to support production of our product and certain of our product candidates and their components. We currently do not have any of our own internal manufacturing capabilities to produce our product 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 ("CMO") to produce custom raw materials, the APIs and finished goods for our product candidates. All of our CMO partners have extensive technical expertise, GMP experience and experience manufacturing our specific technology. Specially, for our commercial DMD program, we have commenced work with our existing manufacturers to increase product capacity from mid-scale to large-scale.

While there are 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 product, based on our diligence to date, we believe our current network of manufacturing partners are able to fulfill all of these requirements, and are capable of continuing to expand capacity as needed. Additionally, we have, and will continue to evaluate further relationships with additional suppliers to increase overall capacity as well as further reduce risks.

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

EXONDYS 51 is 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 EXONDYS 51 to hospitals and hospital outpatient clinics.

Material Agreements

We believe that our RNA-targeted technologies could be broadly applicable for the potential development of pharmaceutical products in many therapeutic areas. To 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 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.

Summit (Oxford) Ltd.

On October 3, 2016, we entered into the exclusive Collaboration and License Agreement (the "Collaboration Agreement") with Summit which grants us the exclusive right to commercialize products in Summit's utrophin modulator pipeline in the E.U., Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States (the "Licensed Territory").

Under the terms of the Collaboration Agreement, we made an up-front payment of $40.0 million to Summit, with the potential for additional payments of up to $192.0 million based on achievement of certain development and regulatory milestones for ezutromid. For each of Summit's future generation small molecule utrophin modulators, we may be required to make up to $290.0 million in development and regulatory milestone payments. Additionally, on a product-by-product basis, we may be required to make up to $330.0 million in sales milestone payments.

The Collaboration Agreement also grants us an option to expand the Licensed Territory. If we exercise this option, then we will be liable for a one-time $10.0 million option fee as well as up to $7.0 million in regulatory milestone payments. For each of Summit's lead product candidate, ezutromid, for the treatment of DMD, and its second generation and future generation small molecule utrophin modulators (collectively, the "Licensed Product"), we may be liable for up to $82.5 million in sales milestone payments.

Additionally, we may be required to make tiered royalty payments ranging from a low to high teens percentage of net sales on a product-by-product basis in the Licensed Territory.

Under the Collaboration Agreement, Summit will be solely responsible for all research and development costs for the Licensed Products until December 31, 2017. Thereafter, Summit will be responsible for 55.0% of the budgeted research and development costs related to the Licensed Products in the Licensed Territory, and we will be responsible for 45.0% of such costs. Any costs in excess of 110.0% of the budgeted amount will be borne by the party that incurred such costs. Summit is also obligated to spend a specified minimum amount on the research and development of certain Licensed Products prior to the end of 2019.

Manufacture and Supply of Licensed Products

Under the Collaboration Agreement, Summit has agreed to use commercially reasonable efforts to supply to us active pharmaceutical ingredient, finished drug product and placebo for us to conduct research, development and commercialization activities for the Licensed Products. We will also have the right to establish back up and second source suppliers under certain circumstances.

Commercialization

Under the Collaboration Agreement, we will be solely responsible for all commercialization activities and associated costs relating to the Licensed Products in the Licensed Territory. We have agreed to use commercially reasonable efforts to commercialize the Licensed Products in specified countries within the Licensed Territory and, if the exclusive option to expand the Licensed Territory to include specified countries in South and Central America (the "Option Territory") is exercised, to use commercially reasonable efforts to commercialize the Licensed Products in certain specified countries within the Option Territory.

University of Western Australia

In April 2013, we entered into an agreement with University of Western Australia ("UWA") under which an existing exclusive license agreement between the two parties was amended and restated (the "Amended and Restated UWA License Agreement"). The Amended and Restated UWA License Agreement grants us specific rights to the treatment of DMD by inducing the skipping of certain exons. EXONDYS 51, SRP-4045 and SRP-4053 fall under the scope of the license agreement. Under the Amended and Restated UWA License Agreement, we may be required to make payments of up to $6.0 million in aggregate to UWA based on the successful achievement of certain development and regulatory milestones relating to EXONDYS 51 and up to five additional product candidates. As of the date of this report, a portion of the $6.0 million development and regulatory milestone payments has been made. We may also be obligated to make payments to UWA of up to $20.0 million upon the achievement of certain sales milestones. Additionally, we may be 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 Amended and Restated UWA License Agreement. However, we have the option to purchase future royalties up-front. Under this option, prior to the First Amendment (defined below), we could be required to make a one-time royalty payment of $30.0 million to UWA.

In June 2016, we entered into the first amendment to the Amended and Restated UWA License Agreement (the "First Amendment") with UWA. Under the First Amendment, we were obligated to make an up-front payment of $7.0 million to UWA upon execution of the First Amendment. Under the terms of the First Amendment, UWA has waived rights to certain royalties and amended the timing of certain other royalty payments under the Amended and Restated UWA License Agreement, including lowering the one-time royalty payment that is due by us upon exercise of the option to purchase future royalties up-front. Upon exercise of the option to purchase future royalties up-front, we would still be obligated to make up to $20.0 million in payments to UWA upon achievement of certain sales milestones.

Currently, the latest date on which an issued patent covered by our agreement with UWA expires is November 2030 (not accounting for any patent term extension, supplemental protection certificate or pediatric extensions that may be available), however, patents granting from pending patent applications could result in a later expiration date.

Strategic Alliances

In connection with our multi-front battle against DMD, we entered into a number of partnering opportunities. We believe these collaborations, taken along with our own programs, represent the most comprehensive approach to treating DMD.

Nationwide Children's Hospital

In January 2016, we entered into an exclusive license agreement with Nationwide Children's Hospital for their Galgt2 gene therapy program. This program explores the potential surrogate gene therapy approach to DMD. Under this approach, the gene therapy looks to induce genes that make proteins that can perform a similar function as dystrophin, with the goal of producing a muscle cell that can function normally even when dystrophin is absent. This approach has the potential to be used broadly in several muscular dystrophies, regardless of their mutation.

In addition, in December 2016, we entered into a research and option agreement with Nationwide Children's Hospital on their microdystrophin gene therapy program. The initial trial, which is expected to go into Phase 1/2a trial in late 2017, will be conducted at Nationwide Children's Hospital. Parent Project Muscular Dystrophy has committed $2.2 million to the trial, with support from additional Duchenne foundations and families. We have committed to the trial through a research agreement with Nationwide Children's Hospital, and we have an exclusive option to exclusively license the program.

Catabasis

In September 2016, we entered into a joint research collaboration with Catabasis Pharmaceuticals, Inc. ("Catabasis") to explore a combination drug treatment approach for DMD. We will contribute our expertise to study an exon skipping treatment while Catabasis will contribute its oral NF-kB inhibition treatment for this combination study for DMD. The objective of the joint research is to study the safety and efficacy of combining these two treatment strategies using a mouse model of DMD, including evaluating the potential for additional or synergistic benefits.

Charley's Fund Agreement

In October 2007, Charley's Fund, Inc. ("Charley's Fund"), a nonprofit organization that funds drug development and discovery initiatives specific to DMD, awarded us a research grant of approximately $2.5 million and, in May 2009, the grant authorization was increased to a total of $5.0 million. Pursuant to the related sponsored research agreement, the grant was provided to support the development of product candidates related to exon 50 skipping using our proprietary exon-skipping technologies. As of December 31, 2016, Charley's Fund had made payments of approximately $3.4 million to us. Revenue associated with this research and development arrangement is recognized based on the proportional performance method. To date, we have recognized approximately $0.1 million as revenue. We have deferred $3.3 million of previous receipts, which are anticipated to be recognized as revenue upon resolution of outstanding performance obligations.

Previously, we noted unexpected toxicology findings in the kidney as part of our series of preclinical studies for AVI-5038, our PMO-based candidate designed for the treatment of individuals with DMD who have an error in the gene coding for dystrophin that can be treated by skipping exon 50. We have conducted additional preclinical studies and have not alleviated the toxicity problem. Pursuant to the terms of our agreement with Charley's Fund, the receipt of additional funds is tied to the satisfaction of certain clinical milestones. Because of the toxicity issues with AVI-5038, satisfaction of the additional milestones under the agreement is unlikely and we do not expect to receive any additional funds from Charley's Fund.

Patents and Proprietary Rights

Our success depends in part upon our ability to protect our core technologies and intellectual property. To accomplish this, we rely on a combination of intellectual property rights, including patents, trade secrets, copyrights and trademarks, as well as regulatory exclusivity and contractual protections.

Our patents and patent applications are directed to our product candidates as well as to our PMO-based technologies platform. We seek patent protection for certain of our product candidates and proprietary technologies by filing patent applications in the U.S. and other countries as appropriate.

Our product candidates and our technologies are primarily protected by composition of matter and use patents and patent applications. Currently, our exon skipping clinical product candidates for DMD include EXONDYS 51 (eteplirsen), SRP-4045 and SRP-4053.

We own patents and have exclusively licensed patents from UWA that provide primary patent protection for eteplirsen, SRP-4045 and SRP-4053 as follows:

Eteplirsen

Patent Number	Country/Region**	Patent Type	Expiration Date*	Owner
U.S. 7,807,816****	United States	Composition of Matter	February 23, 2026	UWA
U.S. 7,960,541****	United States	Composition of Matter	June 28, 2025	UWA
U.S. 8,486,907*****	United States	Methods of Use	June 28, 2025	UWA
U.S. 9,018,368	United States	Composition of Matter	June 28, 2025	UWA
U.S. 9,416,361	United States	Composition of Matter	May 4, 2021	Sarepta
U.S. 9,506,058	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

SRP-4045

Patent Number	Country/Region**	Patent Type	Expiration Date*	Owner
U.S. 8,524,880	United States	Composition of Matter & Methods of Use	April 2, 2026	UWA
U.S. 9,447,415	United States	Composition of Matter	June 28, 2025	UWA
U.S. 9,228,187	United States	Composition of Matter	November 12, 2030	UWA

U.S. 9,416,361	United States	Composition of Matter	May 4, 2021	Sarepta

SRP-4053

Patent Number	Country/Region**	Patent Type	Expiration Date*	Owner
U.S. 8,455,636***	United States	Composition of Matter & Methods of Use	June 28, 2025	UWA
U.S. 9,024,007	United States	Composition of Matter	June 28, 2025	UWA
U.S. 9,416,361	United States	Composition of Matter	May 4, 2021	Sarepta
EP 2 206 781 B1******	Europe	Composition of Matter & Methods of Use	June 28, 2025	UWA

***** Stated expiration dates do not account for any patent term extension, supplemental protection certificate or pediatric extensions that may be available.
**Granted patents in the United States and Europe (EP) are shown here. Additional patent protection in the U.S., Europe (EP) or other countries or regions through pending or granted foreign counterparts may be available.
*** 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 pending (Case No. 2016-2262).
**** Involved in U.S. Patent Interference No. 106,008. Judgement 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 pending (Case No. 2017-1078).
*****Involved in U.S. Patent Interference No. 106,013. Judgment dated September 29, 2015 ordered cancellation of U.S. 8,486,907 to us (UWA). Decision dated December 29, 2015 denied our Request for Rehearing. Appeal by us (UWA) to the Court of Appeals for the Federal Circuit pending (Case Nos. 2016-1937, 2016-2086 (consolidated)).
******Involved in Opposition proceedings initiated on August 25, 2016.

In addition to the foregoing patents that protect eteplirsen, SRP-4045 and SRP-4053, we either solely own or exclusively license from UWA patents and patent applications in the U.S. and in major foreign markets that provide additional protection for eteplirsen as well as our DMD follow-on exon-skipping candidates (e.g., SRP-4045 and SRP-4053), which cover the composition of matter, preparation and/or uses of these drug candidates. These patents, and patent applications, if granted, expire between 2025 and 2036, such expiration dates not accounting for any patent term extension, supplemental protection certificate or pediatric extensions that may be available.

We separately own patents and patent applications in the U.S. and in major foreign markets that cover our proprietary PMO-based technologies (e.g., PPMO, PMOplus®, PMO-X®). These patents, and patent applications, if granted, expire between 2024 and 2036, such expiration dates not accounting for any patent term extension, supplemental protection certificate or pediatric extensions that may be available. We are the owner of multiple federal trademark registrations in the U.S. including, but not limited to, Sarepta®, Sarepta Therapeutics®, PMO*plus*®, PMO-X® the Sarepta Therapeutics logo, EXONDYS®, and EXONDYS 51®. In addition, we have multiple pending trademark applications in the U.S. and in major foreign markets.

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection for our product candidates, and successfully defending these patents against third-party challenges. Our ability to protect our product candidates from unauthorized making, using, selling, offering to sell or importing by third parties is dependent on the extent to which we have rights under valid and enforceable patents that cover these activities.

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.

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. 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. In addition, 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. For example, we are aware of certain claims that our competitor BioMarin has rights to in the U.S that, if granted, may provide the basis for BioMarin or other parties that have rights to these claims to assert that our drug candidates, eteplirsen and/or SRP-4053, infringe on such claims. In 2014, the Patent Trial and Appeal Board ("PTAB") of the USPTO declared various patent interferences between certain patents held by Sarepta under a license from the UWA and patent applications held by BioMarin under license from Academisch Ziekenhuis Leiden ("AZL") related to exon 51 and exon 53 skipping therapies designed to treat DMD. Patents held or licensed to Sarepta and included in these interference proceedings are presumed valid by statute for the duration of these proceedings and any appeals. These interferences have not changed our plans to seek regulatory approval for eteplirsen in foreign markets and pursue post-marketing commitments in the U.S., continue with our clinical development plans for eteplirsen and SRP-4053 or continue to commercialize EXONDYS 51 in the U.S.; however, if final resolution of these interferences and related appeals are not in our favor, our current business, development and commercialization plans for eteplirsen and SRP-4053 may be negatively impacted. For details on and risks related to the interferences that the PTAB has declared involving our patents, please read Risk Factors—*Risks Relating to Our Business—Our success, competitive position and future revenue, if any, depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our technologies, product, and product candidates, 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 pharmaceutical, biotechnology and other life sciences patent situation outside the U.S. can be even more uncertain. For example, BioMarin has rights to European Patent No. EP 1619249. We opposed this patent in the Opposition Division of the European Patent Office ("Opposition Division"), and in November 2011, we announced that, although we succeeded in invalidating some of the patent's claims, the Opposition Division maintained in amended form certain claims of this patent relating to the treatment of DMD by skipping dystrophin exons 51 and 46. We and BioMarin both appealed this decision in June 2013; however, pending final resolution of this matter, the patent at issue may provide the basis for BioMarin or other parties that have rights to such patent in the relevant European country to assert that our drug candidate, eteplirsen, infringes on such patent upon launching eteplirsen in such relevant European country. The outcome of the appeal cannot be predicted or determined as of the date of this report. If, as part of any appeal before the European Patent Office, we are unsuccessful in invalidating BioMarin's claims that were maintained by the Opposition Division or if claims previously invalidated by the Opposition Division are restored on appeal, our ability to commercialize both eteplirsen and other therapeutic candidates, such as SRP-4045 and SRP-4053 could be materially impaired. Moreover, our ability to commercialize eteplirsen could be materially impaired in a European country where BioMarin has a patent related to EP 1619249 while the appeal process remains ongoing before the European Patent Office Board of Appeals. In addition, we are aware of various divisional applications relating to EP 1619249 that are being pursued by BioMarin, which are pending, granted and, in some cases, are proceeding to grant. One of these divisional applications that has been granted claims exon 45 skipping antisense oligonucleotides (EP 2594641) and another claims exon 53 skipping antisense oligonucleotides (EP 2602322). We opposed EP 2594641 and EP 2602322 in the Opposition Division on September 29, 2016 and December 1, 2016, respectively. Should we be unsuccessful in invalidating these patents, or any related patents that grant, our ability to commercialize eteplirsen or our other therapeutic candidates, such as SRP-4045 and SRP-4053, could be materially impaired.

In addition to government, court and regulatory patent decisions, changes in either the patent laws or in interpretations of patent laws in the U.S. and other countries may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be allowed or enforced in the patents that we own or have licensed from/to third-parties. Further, since publication of discoveries in scientific or patent literature often lags behind actual discoveries, there is no assurance that we were the first creator of inventions covered by our pending patent applications, or that we were the first to file patent applications for these inventions.

Government Regulation

The testing, manufacturing, labeling, advertising, promotion, distribution, 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 and its 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 new drug

applications ("NDAs"), 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, first and foremost, submit clinical data providing substantial evidence of safety and efficacy of the product 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:

- preclinical laboratory tests and animal toxicity testing;
- submission of an NDA for conducting human clinical testing to the FDA, which must become effective before human clinical trials commence;
- adequate and well-controlled human clinical trials to establish the safety and efficacy of the drug product for each indication, including placebo-controlled studies or comparison of treated group from clinical trials to data from natural history data or studies;
- 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 NDA and also potentially the nonclinical manufacturing site(s) in the form of pre-approval inspections; and
- FDA review and approval of the NDA.

Preclinical studies 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 preclinical tests and formulation of the compounds for testing must comply with federal regulations and requirements. The results of the preclinical studies, 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 approximately 30 days after receipt by the FDA, unless the FDA raises concerns or questions about the supportive data, or the 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, 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 in accordance with the FDA's Good Clinical Practice ("GCP") requirements and federal and state laws and regulations protecting study subjects. Further, each clinical trial must be reviewed and approved by the Institutional Review Board ("IRB") at or servicing each institution in which the clinical trial will be conducted. The IRB will consider, among other things, rationale for conducting the trial, clinical trial design, participant informed consent, ethical factors, the safety and rights of human subjects and the possible liability of the institution. The FDA can temporarily or permanently halt a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial subjects. The IRB may also require the clinical trial at a particular site be halted, either temporarily or permanently, for failure to comply with GCP or the IRB's requirements, or may impose other conditions.

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 studies. 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 less

than 100 subjects and are conducted in healthy adult volunteers unless the drug is toxic (*e.g.,* cytotoxics) 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. If an investigational drug is found to be potentially effective and to have an acceptable safety profile in early phase studies, 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 an NDA. Phase 3 studies usually include several hundred to several thousand patients for larger, non-orphan drug indications/diseases. However, for orphan drug indications due to their lower prevalence, clinical trials for rare or orphan diseases generally have fewer patients. For these orphan diseases, a company may also try to demonstrate efficacy and safety by comparing treated patients in clinical trials to untreated populations in placebo-controlled clinical trials or to data from natural history studies.
- Phase 4. Phase 4 trials are clinical studies 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. The mandatory studies are used to confirm clinical benefit in the case of drugs approved under the accelerated approval regulations or to provide additional clinical safety or efficacy data for "full" approvals. Failure to promptly conduct and complete mandatory Phase 4 clinical trials could result in withdrawal of approval for products approved under accelerated approval regulations.

A company seeking marketing approval for a new drug in the U.S. must submit to the FDA the results of the preclinical and clinical trials, together with, among other things, detailed information on the manufacture and composition of the product candidate and proposed labeling, in the form of an NDA, including payment of a user fee unless the submission is for an Orphan Indication. The FDA assesses all submitted NDAs for completeness before it accepts them for filing. In some case, the FDA may request additional information before accepting an NDA for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. 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 NDA and respond to the applicant, and six months for a priority NDA. The FDA does not always meet its PDUFA goal dates for standard or priority NDAs. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the NDA 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 recommendation as to whether the application should be approved. Though the FDA is not bound by such recommendations, it considers them carefully when making decisions. If the FDA's evaluations of the NDA and the clinical and manufacturing procedures and facilities are favorable, the FDA may issue an approval letter. If the FDA finds deficiencies in the NDA, it may issue a complete response letter, which defines the conditions that must be met in order to secure final approval of the NDA. If and when those conditions have been met to the FDA's satisfaction, the FDA will issue an approval letter, authorizing commercial marketing of the drug. Sponsors that receive a complete response letter may submit to the FDA information that represents a complete response to the issues identified by the FDA. Resubmissions by the NDA sponsor in response to a complete response letter trigger new review periods of varying length (typically two to six months) based on the content of the resubmission. If the FDA's evaluation of the NDA and the commercial manufacturing procedures and facilities is not favorable, the FDA may refuse to approve the NDA.

A sponsor may also seek designation of its drug candidates under programs designed to accelerate the FDA's review and potential approval of NDAs. For instance, a sponsor may seek FDA designation of a drug candidate as a "fast track product." 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 an NDA before the application is complete. This "rolling review" is available if the applicant provides, and the FDA approves, a schedule for the remaining information. Eteplirsen was granted fast track status in 2007 and both AVI-7288 and AVI-7537 were granted fast track status in September 2012.

The Food and Drug Administration Safety and Innovation Act ("FDASIA") enacted and signed into law in 2012 amended the criteria for the fast track and accelerated approval pathways and, as a result, the pathways now share many common eligibility criteria. FDASIA provides both the sponsor companies and the FDA with greater flexibility and expedited regulatory mechanisms. The statute clarifies that a fast track product may be approved pursuant to an accelerated approval (Subpart – H) or under the traditional approval process. In addition, FDASIA codified the accelerated approval pathway as separate and apart from the fast track pathway, meaning that for drugs to be eligible for accelerated approval, they do not need to be designated under the fast track pathway. FDASIA

reinforces the FDA's authority to grant accelerated approval of a drug that treats a serious condition and generally provides a meaningful advantage over available therapies and demonstrates an 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. FDASIA retains this requirement and further requires those studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical benefit.

Additionally, FDASIA established a new, expedited regulatory mechanism referred to as breakthrough therapy designation. Breakthrough therapy designation, fast track, and accelerated approval are not mutually exclusive and are meant to serve different purposes. The 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.

Finally, 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 NDA 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 NDA 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; and
- continue to have quality control and manufacturing procedures conform to cGMP after 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 NDA, including withdrawal of the product from the market.

Orphan Drug Designation and Exclusivity in the U.S.

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. An orphan drug designation 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. Safety and efficacy of a compound must be established through adequate and well-controlled studies. If a product which has an orphan drug designation subsequently receives FDA approval for the indication for which it has such designation, the product is generally entitled to an orphan drug exclusivity period, which means the FDA may not grant approval to any other application to market the same chemical entity 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. 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. In the U.S., we have been granted an orphan drug designation for AVI-7288 for prophylaxis following documented or suspected exposure to Marburg virus, AVI-7537 for prophylaxis for patients following documented or suspected exposure to Ebola virus and AVI-5038 for the treatment of DMD in patients with a deletion, duplication or frame shift mutation correctable by skipping of exon 50 of the dystrophin gene to allow restoration of the reading frame (including mutations within exons 51, 51-53, or 51-55) in the U.S. The FDA granted orphan drug exclusivity to EXONDYS 51 through September 19, 2023, which is seven years from its approval date, September19, 2016, for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

Distinct from orphan drug exclusivity, the FDA may provide six months of pediatric exclusivity to a sponsor of an NDA, if the sponsor conducted a pediatric study or studies of such 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 new chemical entity exclusivity.

Foreign Regulatory Requirements

We are pursuing regulatory approval of eteplirsen in jurisdictions outside of the U.S. On December 19, 2016, we announced that the EMA validated for centralized assessment our previously submitted MAA for eteplirsen to treat DMD amenable to exon 51 skipping. Thus, in addition to regulations in the U.S., our business will be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products. Irrespective of whether it is an FDA approved drug or an investigational drug, approvals by the comparable regulatory authorities of foreign countries are required before we can commence clinical trials or marketing of the product in those countries. For example, in the EU, the conduct of clinical trials is governed by the currently applicable Clinical Trials Directive 2001/20/EC concerning conduct of clinical trials in the EU and the Directive 2005/28/EC laying down the principles and guidelines on GCP, a system for the approval of clinical trials that has been implemented through national legislation in the member states in the EU. Under this system, a sponsor must obtain approval from the competent national authority of an EU member state in which the clinical trial is to be conducted, or in multiple member states if the clinical trial is to be conducted in a number of countries. Furthermore, the sponsor may only start a clinical trial at a specific study site after the competent ethics committee has issued a favorable opinion. The Clinical Trials Application ("CTA") must include the supporting information prescribed by Directive 2001/20/EC and Directive 2005/28/EC and corresponding national laws of the member states and further detailed in the applicable guidance documents.

In April 2014, the EU adopted a new Clinical Trials Regulation (EU) No 536/2014 to replace the current Clinical Trials Directive. The new Clinical Trials Regulation has come into force, but will come into application in all EU Member States in October 2018 without the need for any national implementing legislation. The new regulation provides an overhaul of the system to ensure greater consistency in the approval of clinical trials with the highest standards of patient safety in the EU. Specifically, the new legislation seeks to simplify and streamline approval. Under the new coordinated procedure for the approval, the sponsor of a clinical trial is required to submit a single application to a reporting EU Member State. The reporting Member State will consult and coordinate with all other Member States in which the clinical trial is planned to be conducted (concerned Member States). If the application is rejected, it can be amended and resubmitted through a central EU Portal. If an approval is issued, the sponsor can start the clinical trial in all concerned Member States. However, a concerned Member State can in certain cases declare an "opt-out" from the approval. In such a case, the clinical trial cannot be conducted in those Member State(s). The Clinical Trials Regulation also aims to streamline and simplify the rules on safety reporting for clinical trials.

In order to obtain marketing authorization for a medicinal product in the EU, applicants are required to submit an MAA to either the national Competent Authorities or the EMA for the Centralized authorization procedures, using the ICH Common Technical Document ("CTD"). Applicants are required to demonstrate the quality, safety and efficacy of the medicinal product in the application for marketing authorization. This includes the requirement to conduct human clinical trials to generate the necessary clinical data. Submission of data in compliance with an agreed PIP is essential for the validation or acceptance of an MAA for review. Medicinal products are authorized in the EU through one of several different procedures, either by the national competent authorities of the EU Member States (through the decentralized, mutual recognition, or national procedures), or through the centralized authorization procedure administered by the EMA. Regulation (EC) No 726/2004 of the European Parliament and of the Council lays down the centralized procedure for the authorization of medicinal products, for which there is a single application, a single evaluation and a single approval allowing direct access to the single market of the EU. Approval via the Centralized Procedure is a two-step process whereby the Committee for Medicinal Products for Human Use ("CHMP") first adopts an "Opinion" recommending grant of a marketing authorization following a review of the submitted data to inform an assessment of benefit/risk. The adopted Opinion can be positive or negative. A positive CHMP opinion is followed by European Commission ("EC") binding decision to grant a marketing authorization. The marketing authorization is valid throughout the EU and is automatically recognized in three of the four European Free Trade Association states (Iceland, Liechtenstein and Norway). These countries collectively belong to the European Economic Area. The timeframe for the evaluation of a marketing authorization application leading to the CHMP opinion is 210 days (discounting procedural clock-stops) from receipt of a valid application for marketing authorization. This time period to complete the scientific review is generally longer than the 210 days as "clock stops" are required to respond to additional written or oral information requested by the EMA. Following a positive CHMP opinion, the EU Commission has 67 days to issue the EC Decision (i.e. the marketing authorization).

Article 3 of Regulation (EC) No 726/2004 defines the scope and eligibility of applications for evaluation under the centralized procedure through which medicinal products must ("mandatory scope") or may ("optional scope" or "Generic/Hybrid") be authorized by the Community. The centralized procedure is compulsory for certain medicinal products, including medicinal products

derived from biotechnological processes, orphan medicinal products, advanced therapy medicinal products and products indicated for the treatment of certain diseases including treatment of neurodegenerative diseases. It is optional for new active substances and products that can demonstrate a significant therapeutic, scientific or technical innovation, where approval would be in the interest of public health. Our portfolio of innovative orphan products for neurodegenerative is subject to the mandatory centralized procedure.

Accelerated evaluation may be granted in exceptional cases, following a justified request from the applicant, when a medicinal product is of a major public health interest, particularly from the point of view of therapeutic innovation. CHMP determines what constitutes a major public interest on a case by case basis. Justifications must include the major benefits expected and present the arguments to support the claim that the medicinal product introduces new methods of therapy or improves on existing methods, thereby addressing, to a significant extent, the greater unmet needs for maintaining and improving public health. During an accelerated assessment, the opinion of the CHMP is given, in principle, within 150 days. The EU Commission Decision is then issued according to the timetable described above.

Innovative medicinal products authorized in the EU on the basis of a full stand-alone marketing authorization application consisting of pharmaceutical and preclinical testing results and clinical trial data (as opposed to an application for a generic marketing authorization that relies on the results of pre-clinical and clinical trials available in the marketing authorization dossier for another, previously approved, reference medicinal product) are treated as reference medicinal products and accordingly entitled to eight years' data exclusivity. During this period, applicants for approval of generics of these innovative products cannot reference or rely upon data contained in the marketing authorization dossier submitted for the innovative medicinal product. Even if the generic product is approved, it cannot be placed on the market until the full 10-year period of market protection has elapsed from the initial authorization of the reference medicinal product. This period can be extended to a maximum of eleven years if, during the first eight years of those ten years, the MA holder for the innovative product obtains an authorization for 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 or as the result of significant preclinical or clinical studies.

In the EU, orphan medicinal product designation is considered by the EMA for drugs intended to diagnose, prevent or treat a life-threatening or very serious condition with a prevalence of no more than 5 in 10,000 people in the EU. In addition, the sponsor is required to demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition that 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. Medicinal products developed for treating serious and chronic conditions would likely not be marketed without incentives due to low market return on the sponsor's development investment. As such, they may also be eligible for an EU orphan drug designation. 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 a new marketing application for a similar medicinal product for the same therapeutic indication as the approved orphan medicinal product. Pursuant to Regulation (EC) 1901/2006 on medicinal products for pediatric use, the 10-year orphan market exclusivity can be extended to a maximum period of 12 years on the satisfactory completion of all the key elements of the agreed PIP. We have been granted orphan drug designation for eteplirsen and AVI-5038 in the EU.

Similarly to the U.S., marketing authorization holders and manufacturers of medicinal products are subject to comprehensive regulatory oversight by the EMA and/or the national competent authorities of the EU Member States. This oversight applies both before and after the granting of manufacturing and marketing authorizations. 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 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: 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 collection and use of personal health data and other personal information in the EU is governed by the provisions of the Data Protection Directive as implemented into national laws by the EU Member States. This Directive imposes a number of strict obligations and restrictions on the ability to collect, analyze and transfer patient data, including sensitive health data from clinical trials and adverse event reporting. There is, moreover, a growing trend towards imposition of an obligation of public disclosure of clinical trial data in the EU, which adds to the complexity of obligations relating to the processing of health data from clinical trials. Such public disclosure obligations are provided in the new EU Clinical Trials Regulation, EMA disclosure initiatives, and voluntary commitments by industry. The Data Protection Directive also includes requirements relating to the consent of the individuals to whom

the personal data relates, the information provided to the individuals prior to processing their personal data or personal health data, notification of data processing obligations to the competent national data protection authorities and the security and confidentiality of the personal data. The Data Protection Directive also prohibits the transfer of personal data to countries outside of the EU Member States that are not considered by the EU to provide an adequate level of data protection. These countries include the U.S. Failure to comply with the requirements of the Data Protection Directive and the related national data protection laws of the EU Member States may result in fines and other administrative penalties. Data protection authorities from the different EU Member States may interpret the EU Data Protection Directive and national laws differently, which adds to the complexity of processing personal data in the EU Guidance developed at both EU level and at national level in individual EU Member States concerning implementation and compliance practices are often updated or otherwise revised.

In December 2015, a proposal for an EU General Data Protection Regulation, intended to replace the current EU Data Protection Directive, was agreed between the European Parliament, the Council of the European Union and the European Commission. The EU General Data Protection Regulation, which was officially adopted in April 2016 and will be applicable in May 2018, will introduce new data protection requirements in the EU, as well as substantial fines for breaches of the data protection rules. The EU General Data Protection Regulation will increase our responsibility and liability in relation to any personal data that we process, and we may be required to put in place additional mechanisms to ensure compliance with the new EU data protection rules.

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.

Expanded / Early Access

In certain countries, drug products approved in the U.S. or the EU 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, providing the product either free of charge on a named patient basis, and providing the product on a compassionate use basis or can be charged at a price agreed by the National Agencies. 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.

Other Regulatory Requirements

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. 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.

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 crime or civil sanctions, including fines and civil monetary penalties, and/or exclusion from federal health care programs (including Medicare and Medicaid). 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 brining suits on behalf of the government under the federal False Claims Act ("FCA"). Violations of international fraud and abuse laws could result in similar penalties, including exclusion from participation in health programs outside the U.S. 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. The Healthcare Reform Act clarifies the intent requirements of the federal Anti-Kickback Statute, providing that a person or entity does not need to have actual knowledge of the statute or a specific intent to violate the statute. Violations of the federal Anti-Kickback Statute can result in exclusion from Medicare, Medicaid or other governmental programs as well as civil and criminal fines and penalties of up to $50,000 per violation and three times the amount of the unlawful remuneration.

In addition, the Healthcare Reform Act revised the FCA to provide that a claim arising from a violation of the Federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA. The majority of states also have anti-kickback, false claims, and similar fraud and abuse laws and although the specific provisions of these laws vary, their scope is generally broad, and there may not be regulations, guidance or court decisions that apply the laws to particular industry practices. There is therefore a possibility that our practices might be challenged under the anti-kickback statutes or similar laws.

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 (which may include claims for services not provided as claimed or claims for medically unnecessary services). False or fraudulent claims for purposes of the FCA carry fines and civil penalties for violations ranging from $10,781 to $21,563 for each false claim, plus up to three times the amount of damages sustained by the federal government and, most critically, may provide the basis for exclusion from federally funded healthcare programs. There is also a criminal FCA statute by which individuals or entities that submit false claims can face criminal penalties. In addition, under the federal Civil Monetary Penalty Law, the Department of Health and Human Services Office of Inspector General has the authority to exclude from participation in federal health care programs or to impose civil penalties against any person who, among other things, knowingly presents, or causes to be presented, certain false or otherwise improper claims. Our activities relating to the sale and marketing of our products may be subject to scrutiny under these laws.

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"). The Healthcare Reform Act requires manufacturers to submit information to the FDA on the identity and quantity of drug samples requested and distributed by a manufacturer during each year. Recent scrutiny of pharmaceutical pricing practices by certain companies may lead to changes in laws that currently allow substantial flexibility in pricing decisions by pharmaceutical manufacturers. Such changes could occur at the federal level or state level and may be adopted by statute, rule, or sub-regulatory policies. State laws may also require disclosure of pharmaceutical pricing information and marketing expenditures. Many of these laws and regulations contain ambiguous requirements or require administrative guidance for implementation. Given the lack of clarity in laws and their implementation, our activities could be subject to the penalty provisions of the pertinent federal and state laws and regulations.

Pharmaceutical Pricing and Reimbursement

Our first commercial product in the U.S., EXONDYS 51, was granted accelerated approval by the FDA on September 19, 2016. We have an ongoing dialogue with payors with the goal of obtaining broad coverage for EXONDYS 51. To date, payors' policies on coverage for EXONDYS 51 have varied on their approach, including policies that allow broad coverage per the EXONDYS 51 prescribing information, policies that provide limited coverage, to policies that have denied coverage. The majority of payors are reviewing coverage on a case-by-case basis or approving with restrictions. Our revenue depends, in part, upon the price that payors, including government authorities or programs, private health insurers and other organizations, reimburse on behalf of patients and physicians for the cost of EXONDYS 51.

Third Party Reimbursement and Pricing in the U.S.

Commercial Insurance. Coverage and reimbursement of our EXONDYS 51 varies from commercial payor to commercial payor. Many commercial payors, such as managed care plans manage access to FDA approved products' coverage partly to control costs to their plans, and may use drug formularies and medical policies to limit their exposure. Exclusion from policies can directly reduce product usage in the payor's patient population and may negatively impact utilization in other payor plans, as well.

Medicaid. Our product EXONDYS 51 is eligible to be reimbursed by Medicaid. Medicaid is a joint federal and state program that is administered by the states for low income and disabled beneficiaries. Under the Medicaid Drug Rebate Program, participating manufacturers are required to pay a rebate for each unit of product reimbursed under the state Medicaid programs. The amount of the rebate for each product is set by law and may be subject to an additional discount if certain pricing increases more than inflation. State Medicaid programs and Medicaid managed care plans can seek additional "supplemental" rebates from manufacturers in connections with favorable positioning on formularies.

Medicare. Medicare is a federal program that is administered by the federal government that covers individuals age 65 and over as well as those with certain disabilities. Our product EXONDYS 51 is eligible for reimbursement under Medicare Part B. Medicare Part B generally covers drugs that must be administered by physicians. Medicare Part B pays for such drugs under a payment methodology based on the average sales price ("ASP") of the drugs. Reimbursement levels and reimbursement methodologies have come under scrutiny and may be subject to change. The Centers for Medicare & Medicaid Services ("CMS") are

also increasingly bundling drug reimbursement into procedure costs, which can severely decrease the reimbursement rates for some manufacturers' drugs.

Federal Purchasers. Drug products are subject to discounted pricing when purchased by federal agencies via the Federal Supply Schedule ("FSS"). FSS participation is required for a drug product to be covered and reimbursed by certain federal agencies and for coverage under Medicaid, Medicare Part B and the Public Health Service ("PHS") 340B drug pricing program. FSS pricing is negotiated periodically with the Department of Veterans Affairs. FSS pricing is intended not to exceed the price that a manufacturer charges its most-favored non-federal customer for its product. In addition, prices for drugs purchased by the Veterans Administration, Department of Defense (including drugs purchased by military personnel and dependents through the TRICARE retail pharmacy program), Coast Guard, and PHS are subject to a cap on pricing (known as the "federal ceiling price") and may be subject to an additional discount if pricing increases more than the rate of inflation.

PHS 340B Drug Pricing Program. To maintain coverage of drugs under the Medicaid Drug Rebate Program and Medicare Part B, manufacturers are required to extend discounts to certain purchasers under the PHS 340B drug pricing program. Purchasers eligible for discounts include hospitals that serve a disproportionate share of financially needy patients, community health clinics and other entities that receive health services grants from the PHS.

ACA. The United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. For example, in March 2010, the U.S. Congress enacted the Affordable Care Act which expanded health care coverage through Medicaid expansion and the implementation of the individual health insurance mandate and which included changes to the coverage and reimbursement of drug products under government health care programs. Modifications to or repeal of all or certain provisions of the Affordable Care Act are expected under the new Trump administration and Congress. We cannot predict the ultimate content, timing or effect of any changes to the Affordable Care Act or other federal and state reform efforts. There is no assurance that federal or state health care reform will not adversely affect our future business and financial results.

General legislative cost control measures may also affect reimbursement for our products. The Budget Control Act, as amended, resulted in the imposition of 2% reductions in Medicare (but not Medicaid) payments to providers in 2013 and will remain in effect through 2025 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.

Third Party Reimbursement and Pricing outside the U.S.

In the EU and certain other territories, price controls and Health Technology Assessments for new, highly priced medicines are expected. Uncertainty exists about the pricing and reimbursement status of newly approved products in the EU. 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. 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 an economically viable reimbursement terms in the EU and other markets will require strong education and awareness efforts around DMD.

Competition

The pharmaceutical and biotechnology industries are intensely competitive, and any product candidate developed by us 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 marketing resources than we have. 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, many of these competitors have significantly greater commercial infrastructures than we have. Our ability to compete successfully will depend largely on:

- our ability to complete clinical development and obtain regulatory approvals for our product candidates;
- the efficacy, safety and reliability of our product candidates;
- the timing and scope of regulatory approvals;
- product acceptance by physicians and other health-care providers;
- protection of our proprietary rights and the level of generic competition;
- the speed at which we develop product candidates;
- our ability to supply commercial quantities of a product to the market;

- obtaining reimbursement for product use in approved indications;
- our ability to recruit and retain skilled employees; and
- the availability of substantial capital resources to fund development and commercialization activities, including the availability of funding from the U.S. government.

DMD Program Competition. Currently, other than EXONDYS 51, no disease-modifying product in the U.S. has been approved for the treatment of DMD and no such product is commercially available outside the European Economic Area ("EEA"). Other companies, however, have product candidates or other interests in development for the treatment of DMD.

PTC Therapeutics ("PTC") has a small molecule candidate, ataluren, which targets nonsense mutations in development. The European Commission granted conditional marketing authorization for ataluren for the treatment of a subset of DMD patients in August 2014. In January 2016, PTC announced the completion of its rolling submission of an NDA for ataluren to the FDA and submission of its Phase 3 Ataluren Confirmatory Trial ("ACT") DMD clinical trial result to the EMA. Ataluren uses a distinct scientific approach that addresses a different genotype of DMD patients compared to eteplirsen. Therefore, we do not believe ataluren is appropriate for the treatment of DMD patients that are amenable to exon-skipping therapy.

BioMarin has an exon 51-skipping product candidate, drisapersen. An NDA for drisapersen was filed by the FDA and an MAA was submitted to the EMA in June 2015. In January 2016, the FDA issued a complete response letter and declined the approval for drisapersen for the treatment of DMD. 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 DMD. BioMarin further announced its intent to continue to explore the development of next generation oligonucleotides for the treatment of DMD.

Wave Life Sciences ("Wave") announced the selection of its stereopure exon-skipping candidate, WVE-210201, to target deletions of Exon 51. WVE-210201 is said to be entering the clinic in the second half of 2017.

Nippon Shinyaku has reported early clinical development data for its exon 53 skipping candidate, NS-065, and the first patient dosed in a Phase 2 study, which is enrolling in the U.S. It has also reported a phase 1/2 study in Japan. NS-065 has been reported to have received an orphan drug designation in the U.S. and was granted Fast Track by FDA.

Daiichi Sankyo has reported early clinical development in Japan for its exon 45 skipping candidate, DS-5141b.

Solid Biosciences, LLC, Bamboo Therapeutics, Inc. and Genethon have reported that they are each in the pre-clinical stages of development of a gene therapy technology for DMD.

Other companies continue to pursue approval of products for the treatment of DMD and their products may or may not prove to be safer and/or more efficacious than the products and product candidates in our DMD pipeline. Regarding any of these competitors, it is unknown if further clinical development of these or other exon-skipping compounds is planned.

Additionally, companies such as Santhera, Summit, Catabasis, Pfizer and Tivorsan have unique product candidates in different stages of development or approval in DMD which we believe could be seen as complementary to exon skipping and not a direct replacement of our clinical candidates at this time.

In addition, several companies and institutions have recently entered into collaborations or other agreements for the development of product candidates, including mRNA, gene (CRISPR, AAV, etc.) or 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 Biogen Inc., Ionis, Alexion Pharmaceuticals, Inc., Sanofi, Shire, Eli Lilly, Alnylam Pharmaceuticals, Inc., Moderna Therapeutics, Inc., Summit, Akashi, Catabasis and Oxford University.

Platform Technology Competition. We believe that other biotechnology and pharmaceutical companies share a focus on RNA-targeted drug discovery and development. Competitors with respect to our RNA-targeted technologies include, but are not limited to, Alnylam, Tekmira Pharmaceuticals Corp., Ionis, BioMarin, Sanofi, Synthena AG, Santaris Pharma A/S (now Roche), Nippon Shinyaku, Daiichi Sankyo and Wave.

Employees

As of December 31, 2016, we had 197 employees, 89 of whom hold advanced degrees. Of these employees, 90 are engaged directly in research and development activities and 107 are in general and administration including 31 in the sales force. None of our employees are covered by collective bargaining agreements and we consider relations with our employees to be good.

Item 1A. Risk Factors.

Factors That Could Affect Future Results

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 EXONDYS 51 in the U.S.; we may not be able to meet expectations with respect to EXONDYS 51 sales or attain profitability and positive cash-flow from operations.

On September 19, 2016, the FDA granted accelerated approval for EXONDYS 51 as a therapeutic treatment for DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 is commercially available. The commercial success of EXONDYS 51 will depend on a number of factors, including, but not limited to:

- the effectiveness of our sales, managed markets and marketing efforts;
- the effectiveness of our commercialization activities, including negotiating and entering into any additional commercial and supply contracts, scaling up manufacturing and hiring any additional personnel;
- FDA-mandated package insert requirements and the time it would take us to comply with any related FDA post-marketing requirements and commitments;
- demonstration and/or confirmation of clinical efficacy and safety and acceptance of the same by the medical community;
- the occurrence of any side effects, adverse reactions or misuse, or any unfavorable publicity in these areas;
- whether EXONDYS 51 can consistently be manufactured in commercial quantities and at acceptable costs;
- the cost-effectiveness of the product;
- the adoption of EXONDYS 51 by physicians, which depends on whether physicians view it as a safe and effective treatment for patients with DMD;
- adequate reimbursement by third parties, including government payors, managed care organizations and private health insurers;
- our ability to comply with the FDA requirements, and achieve the required clinical endpoints in the studies included in the EXONDYS 51 approval letter including our ability to successfully conduct and achieve the endpoints in the two-year post-approval study required by the FDA to verify EXONDYS 51's clinical benefit;
- the need for, and success of, other confirmatory trials and post-marketing requirements;
- the development or commercialization of competing products or therapies for the treatment of DMD, or its symptoms;
- marketing and distribution support for EXONDYS 51;
- our ability to increase awareness of the importance of genetic testing and knowing / understanding DMD mutations, and identifying and addressing procedural barriers to obtaining therapy;
- our ability to remain compliant with laws and regulations that apply to us and our commercial activities;

- the actual market-size for EXONDYS 51, which may be different than expected;
- the sufficiency of our drug supply to meet commercial and clinical demands which could be negatively impacted if our projections on the potential number of amenable patients and their average weight are inaccurate, we are subject to unanticipated regulatory requirements that increase our drug supply needs, our current drug supply is destroyed or negatively impacted at our manufacturing sites, storage sites or in transit, or it takes longer than we project for the number of patients we anticipate to get on EXONDYS 51 and any significant portion of our EXONDYS 51 supply expires before we are able to sell it;
- our ability to obtain regulatory approvals to commercialize EXONDYS 51 in markets outside of the U.S.; and
- the awareness of patients with DMD of their mutation and whether the mutation is amenable to EXONDYS 51.

In addition, the process leading to a patient's first infusion of EXONDYS 51 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. As the launch of EXONDYS 51 progresses, we expect the variation among patients to decline, leading to a faster time to infusion. However, delays in the process prior to first infusion could negatively impact the sales of EXONDYS 51.

We may experience significant fluctuations in sales of EXONDYS 51 from period to period and, ultimately, we may never generate sufficient revenues from EXONDYS 51 to reach or maintain profitability or sustain our anticipated levels of operations.

EXONDYS 51 may cause undesirable side effects or have other properties that could limit its commercial potential.

If we or others identify previously unknown side effects, in particular if they are severe, or if known side effects are more frequent or severe than in the past, then:

- sales of EXONDYS 51 may be modest;
- regulatory approvals for EXONDYS 51 may be restricted or withdrawn;
- we may decide to, or be required to, send product warning letters or field alerts to physicians, pharmacists and hospitals;
- additional non-clinical or clinical studies, changes in labeling or changes to manufacturing processes, specifications and/or facilities may be required;
- our reputation in the marketplace may suffer; and
- government investigations or lawsuits, including class action suits, may be brought against us.

Any of the above occurrences would harm or prevent sales of EXONDYS 51, increase our expenses and impair our ability to successfully commercialize EXONDYS 51. Furthermore, once EXONDYS 51 is commercially available, it may be used in a wider population and in a less rigorously controlled environment than in clinical studies. As a result, regulatory authorities, healthcare practitioners, third-party payors or patients may perceive or conclude that the use of EXONDYS 51 is associated with previously unknown serious adverse effects, undermining our commercialization efforts.

We currently rely on third parties to manufacture EXONDYS 51 and to produce our product candidates; our dependence on these parties, including any inability on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet commercial or clinical product demand may impair the commercialization of EXONDYS 51 and the research and development programs and potential commercialization of our product candidates.

We currently do not have the internal ability to undertake the manufacturing process for EXONDYS 51 or our product candidates in the quantities needed to meet commercial demand for EXONDYS 51, or to conduct our research and development programs and conduct clinical trials for our product candidates. Therefore, 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), drug substance ("API") and drug product, as well as to perform additional steps in the manufacturing process, such as the filling and labeling of vials and storage of EXONDYS 51 and our product candidates. There are a limited number of third parties with facilities and capabilities suited for the manufacturing process of EXONDYS 51 and our product candidates, which creates a heightened risk that we may not be able to obtain materials and APIs in the quantity and purity that we require. In addition, the process for adding new

manufacturing capacity can be lengthy and could cause delays in our development efforts. Any interruption of the development or operation of those facilities due to, among other reasons, events such as order delays for equipment or materials, equipment malfunction, 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 earthquake or fire, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available EXONDYS 51, product candidates or materials.

If these third parties were to 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 EXONDYS 51 or our product candidates in sufficient quality and quantity required for commercial use of EXONDYS 51 and/ or for planned pre-clinical testing, clinical trials and potential commercial use of our product candidates would be adversely affected.

We have, through our third-party manufacturers, produced or are in the process of producing clinical and commercial supply of our product candidates and EXONDYS 51, respectively, based on our current understanding of market demands and our needs for our research and development efforts and clinical trials. In light of the limited number of third parties with the expertise to produce EXONDYS 51 and our 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 manufacturing arrangements on the commercially reasonable terms necessary to provide adequate supply of EXONDYS 51 to meet demands that exceed our commercial assumptions or to provide adequate supply of our product candidates to meet demands that exceed our clinical assumptions. 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 EXONDYS 51 and our 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 could impact our ability to execute our business plans on expected or required timelines in connection with the commercialization of EXONDYS 51 and the continued development of our product candidates. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and/or substantial termination penalties, which could have a material adverse effect on our business prior to and after commercialization.

The third parties we use in the manufacturing process for EXONDYS 51 and our 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 and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations. We do not have control over a third-party manufacturer's compliance with these regulations and requirements. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of EXONDYS 51 and our product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively. The failure to achieve and maintain compliance with cGMP and other applicable government regulations, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in product recalls, patient injury or death. If our contract manufacturers fail to adhere to applicable cGMP and other applicable government regulations, or experience manufacturing problems, we will suffer significant 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 results, we may not be able to successfully commercialize EXONDYS 51.

We may not be able to successfully scale up manufacturing of EXONDYS 51 or our product candidates in sufficient quality and quantity or within sufficient timelines, or be able to secure ownership of intellectual property rights developed in this process, which could negatively impact our commercialization of EXONDYS 51 and or the development of our product candidates.

We are working to increase manufacturing capacity and scale up production of some of the components of our drug products. During the remainder of 2017, our focus remains on (i) achieving larger-scale manufacturing capacity for EXONDYS 51 throughout the manufacturing supply chain and (ii) continuing to increase material and API production capacity to provide the anticipated amounts of drug product needed for our planned studies for our product candidates. We may not be able to successfully increase manufacturing capacity or scale up 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. Compliance with cGMP requirements and other quality issues may arise during our efforts to increase manufacturing capacity and scale up production with our current or any new contract manufacturers. These issues may arise in connection with the underlying materials, the inherent properties of EXONDYS 51 or a product candidate, EXONDYS 51 or a product candidate in combination with other components added during the manufacturing and packaging process or during shipping and storage of the APIs or finished drug product. In addition, in order to release EXONDYS 51 for commercial use and demonstrate

stability of product candidates for use in late stage clinical trials (and any subsequent drug products for commercial use), our manufacturing processes and analytical methods must be validated in accordance with regulatory guidelines. We may not be able to successfully validate, or maintain validation of, our manufacturing processes and analytical methods or demonstrate adequate purity, stability or comparability of EXONDYS 51 or our product candidates in a timely or cost-effective manner, or at all. If we are unable to successfully validate our manufacturing processes and analytical methods or to demonstrate adequate purity, stability or comparability, the commercial availability of EXONDYS 51 and the continued development and/or regulatory approval of our product candidates may be delayed, which could significantly harm our business.

During work with our third-party manufacturers to increase manufacturing capacity and scale up production, it is possible that they could make proprietary improvements in the manufacturing and scale-up processes for EXONDYS 51 or our 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, it is possible that we will 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. Any failure to secure the intellectual rights required for the manufacturing process needed for large-scale clinical trials or commercialization of EXONDYS 51 or the continued development of our product candidates could cause significant delays in our business plans or otherwise negatively impact the commercialization of EXONDYS 51 or the continued development of our product candidates.

If we are unable to maintain our agreements with third parties to distribute EXONDYS 51 to patients, our results of operations and business could be adversely affected.

We will rely on third parties to commercially distribute EXONDYS 51 to patients. We have contracted with a third-party logistics company to warehouse EXONDYS 51 and with specialty pharmacies to sell and distribute it 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. We are also planning to contract with a third-party call center to help us with some or all of the following: coordinate prescription intake and distribution, reimbursement adjudication, patient financial support, and ongoing compliance support. This distribution network will require 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 EXONDYS 51. If we are unable to effectively manage the distribution process, the commercial launch and sales of EXONDYS 51, 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 specialty pharmacies and a call center 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 EXONDYS 51 or serious adverse events and/or product complaints regarding EXONDYS 51;
- not effectively sell or support EXONDYS 51;
- reduce or discontinue their efforts to sell or support EXONDYS 51;
- not devote the resources necessary to sell EXONDYS 51 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 and lower product revenue, which would harm our results of operations and business.

If we are unable to successfully maintain and further develop internal commercialization capabilities, sales of EXONDYS 51 may be negatively impacted.

We have hired a commercial team and put in the organizational infrastructure we believe we need for a successful commercial launch of EXONDYS 51. We will need to commit significant time and financial and managerial resources to maintain and further develop our marketing and sales force to ensure they have the technical expertise required to address any challenges we may face with the commercialization of EXONDYS 51. Factors that may inhibit our efforts to maintain and develop our commercialization 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 EXONDYS 51, to deliver a consistent message regarding EXONDYS 51 and be effective in convincing physicians to prescribe EXONDYS 51;

- an inability to equip sales personnel with effective materials, including medical and sales literature to help them educate physicians and our healthcare providers regarding EXONDYS 51 and its proper administration;

- unforeseen costs and expenses associated with maintaining and further developing an independent sales and marketing organization.

If we are not successful in establishing and maintaining an effective sales and marketing infrastructure, we will have difficulty commercializing EXONDYS 51, which would adversely affect our business and financial condition.

We are subject to uncertainty relating to reimbursement policies which, if not favorable for EXONDYS 51, could hinder or prevent EXONDYS 51's commercial success.

Our ability to successfully commercialize EXONDYS 51 in the U.S. will depend 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 prices charged for medical products and services. We may not obtain adequate third-party coverage or reimbursement for EXONDYS 51, or we may be required to sell EXONDYS 51 at an unsatisfactory price.

We expect that private insurers will consider the efficacy, cost-effectiveness and safety of EXONDYS 51 in determining whether to approve reimbursement for EXONDYS 51 and at what level. We continue to have discussions with payors, some of which may eventually deny coverage. We may not receive approval for reimbursement of EXONDYS 51 from private 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 do not receive these approvals on a timely basis. Our business could also be adversely affected if private insurers, including managed care organizations, the Medicare or Medicaid programs or other reimbursing bodies or payors limit the indications for which EXONDYS 51 will be reimbursed.

Additionally, in the wake of government and public scrutiny of pharmaceutical pricing practices, there have been efforts at the federal and state levels to implement legislation or regulations to promote transparency in drug pricing or limit drug prices. Such initiatives are likely to continue the pressure on pharmaceutical pricing, may require us to modify our business practices with healthcare practitioners, and may also increase our regulatory burdens and operating costs.

In some foreign countries, particularly Canada and the countries of Europe, the pricing of prescription pharmaceuticals is subject to strict governmental control. In these countries, pricing negotiations with governmental authorities can take six to 12 months or longer after the receipt of regulatory approval and product launch. To obtain favorable reimbursement for the indications sought or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our products, including EXONDYS 51, to other available therapies. Furthermore, several European countries 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.

We expect to experience pricing pressures in connection with the sale of EXONDYS 51 and our future products due to the 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.

Healthcare reform and other governmental and private payor initiatives may have an adverse effect upon, and could prevent commercial success of EXONDYS 51 and our other product candidates.

The U.S. government and individual states are aggressively pursuing healthcare reform, as evidenced by the passing of the Affordable Care Act. The Affordable Care Act substantially changed the way healthcare is financed by both governmental and private insurers and contains a number of provisions that affect coverage and reimbursement of drug products and/or that could potentially reduce the demand for pharmaceutical products such as increasing drug rebates under state Medicaid programs for brand name prescription drugs and extending those rebates to Medicaid managed care and assessing a fee on manufacturers and importers of brand name prescription drugs reimbursed under certain government programs, including Medicare and Medicaid. Other aspects of healthcare reform, such as expanded government enforcement authority and heightened standards that could increase compliance-related costs, could also affect our business. Modifications to or repeal of all or certain provisions of the Affordable Care Act are expected as a result of the outcome of the recent presidential election and Republicans maintaining control of Congress, consistent

with statements made by Donald Trump and members of Congress during the presidential campaign and following the election. We cannot predict the ultimate content, timing or effect of any changes to the Affordable Care Act or other federal and state reform efforts. 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 reform 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 healthcare costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. We anticipate that the U.S. Congress, state legislatures and the private sector will continue to consider and may adopt healthcare policies intended to curb rising healthcare costs. These cost containment measures may include:

- controls on government funded reimbursement for drugs;
- caps or mandatory discounts under certain government sponsored programs;
- controls on healthcare providers;
- challenges to the pricing of drugs or limits or prohibitions on reimbursement for specific products through other means;
- reform of drug importation laws;
- expansion of use of managed care systems in which healthcare providers contract to provide comprehensive healthcare for a fixed cost per person; and
- prohibition on direct-to-consumer advertising or drug marketing practices.

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 EXONDYS 51 and our other potential products, which would have an adverse effect on our net revenues and operating results.

The Food and Drug Administration Amendments Act of 2007 also provides the FDA enhanced post-marketing authority, including the authority to require post-marketing studies and clinical trials, labeling changes based on new safety information, and compliance with risk evaluations and mitigation strategies approved by the FDA. The FDA's exercise of this authority could result in increased development-related costs following the commercial launch of EXONDYS 51, and could result in potential restrictions on the sale and/or distribution of EXONDYS 51, even in its approved indications and patient populations.

Even though EXONDYS 51 has been approved by the FDA as a treatment for DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping, it faces future post-approval development and regulatory requirements, which will present additional challenges.

On September 19, 2016, the FDA granted accelerated approval for EXONDYS 51 as a therapeutic treatment for patients with DMD who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. This indication is based on an increase in dystrophin in skeletal muscles observed in some patients treated with EXONDYS 51. EXONDYS 51 will be subject to ongoing FDA requirements governing the labeling, packaging, storage, advertising, promotion, recordkeeping and submission of safety, efficacy and other post-market information.

Continued approval for this indication is contingent upon completing various post-marketing requirements and commitments, including the requirement to conduct a randomized, controlled clinical trial to verify the drug's clinical benefit. 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 EXONDYS 51; and could negatively impact our financial results. Failure to meet post-approval commitments and requirements, including completion of enrollment and obtaining positive safety and efficacy data from our confirmatory studies for EXONDYS 51, would lead to negative regulatory action from the FDA and/or withdrawal of regulatory approval of EXONDYS 51.

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 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 require labeling changes implementation of risk evaluation and mitigation strategy (REMS) program. 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, or suspension of manufacturing. If we or the manufacturing facilities for EXONDYS 51 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.

Even though EXONDYS 51 has been approved for marketing in the U.S., we may never receive approval to commercialize EXONDYS 51 outside of the U.S.

We are not permitted to market or sell EXONDYS 51 in the EU or in any other foreign countries on a commercial basis until we receive the requisite approval from such country's regulatory authorities. In order to market any product in a foreign country, 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. 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. 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 EXONDYS 51.

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 eteplirsen and could adversely affect our business and financial condition. Any such complications may reduce our target market and delay or limit the full commercial potential of eteplirsen. Many foreign countries are undertaking cost-containment measures that could affect pricing or reimbursement of eteplirsen.

In November 2016, we submitted an MAA for eteplirsen to the EMA. The application was validated in December 2016 and is currently under review. We believe that we submitted a robust package of clinical, dystrophin and safety data to support the review of eteplirsen. We also believe that, in contrast to the FDA approval, the clinical data will be central in evaluating the application, while dystrophin will be supportive of the drug's mechanism of action. However, obtaining approval of an MAA or any other filing for approval in a foreign country is an extensive, lengthy, expensive and uncertain process, and the regulatory authority may reject a filing or delay, limit or deny approval of eteplirsen for many reasons, including:

- we may not be able to demonstrate to the satisfaction of foreign regulatory authorities that eteplirsen is safe and effective for the treatment of patients with DMD who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping;
- the results of clinical trials may not meet the level of statistical or clinical significance required for approval by foreign regulatory authorities;

- foreign regulatory authorities 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 may not find the data from clinical trials sufficient to demonstrate that eteplirsen's clinical benefits outweigh its safety risks; or such regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials and require that we conduct one or more additional trials;
- regulatory authorities outside the U.S. may not accept data generated at our clinical trial sites;
- regulatory authorities outside the U.S. may impose limitations or restrictions on the approved labeling of eteplirsen, 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;
- we may not be able to validate our manufacturing process to the satisfaction of regulatory authorities outside the U.S. or demonstrate adequate cGMP compliance; or
- regulatory authorities outside the U.S. may adopt new or revised approval policies and regulations.

If we are unable to execute effectively our sales and marketing activities outside the U.S., we may be unable to generate sufficient product revenue.

EXONDYS 51 is our first commercial product. As a result, we have only recently had to build our sales, marketing, managerial and other non-technical capabilities in the U.S. We plan to continue to build commercial infrastructure in the EU and in other key countries in order to be ready to launch eteplirsen with a relatively small specialty sales force if the product is ultimately approved in those jurisdictions. The establishment and development of our commercial infrastructure will continue to be expensive and time consuming, and we may not be able to successfully fully develop this capability in a timely manner or at all. We anticipate developing a commercial infrastructure across multiple jurisdictions, if eteplirsen is approved in such jurisdictions. Doing so will require a high degree of coordination and compliance with laws and regulations in such jurisdictions. If we are unable to effectively coordinate such activities or comply with such laws and regulations, our ability to commercialize eteplirsen in such jurisdictions will be adversely affected. Even if we are able to effectively hire a sales force and develop a marketing and sales infrastructure, our sales force may not be successful in commercializing eteplirsen or any other product candidate that we develop. If we are unable to establish adequate manufacturing, sales, marketing, supply and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable.

EXONDYS 51 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 EXONDYS 51, particularly in the near term in the U.S., depends upon its level of market adoption by patients, payors and healthcare providers. If EXONDYS 51 does not achieve an adequate level of market adoption for any reason, our potential profitability and our future business prospects will be severely adversely impacted. The degree of market acceptance of EXONDYS 51 depends on a number of factors, including:

- our ability to demonstrate to the medical community, including specialists who may purchase or prescribe EXONDYS 51, the clinical efficacy and safety of EXONDYS 51 as the prescription product of choice DMD amenable to exon-51 skipping in the U.S.;
- the effectiveness of our sales and marketing organizations and distribution networks;
- the ability of patients or providers to be adequately reimbursed for EXONDYS 51 in a timely manner from government and private payors;
- the actual and perceived efficacy and safety profile of EXONDYS 51, particularly if unanticipated adverse events related to EXONDYS 51 treatment arise and create safety concerns among potential patients or prescribers; and
- the efficacy and safety of competitive therapies.

The patient population suffering from DMD, and in particular those with mutations amenable to exon-51 skipping, 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.

DMD is a fatal genetic neuromuscular disorder affecting 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. Our estimate of the size of the patient population is

based on published studies as well as internal analyses. If the results of these studies or our analysis of them do not accurately reflect the number of patients with DMD, our assessment of the market may be inaccurate, making it difficult or impossible for us to meet our revenue goals, or to obtain and maintain profitability. Since EXONDYS 51 targets a small patient population, 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 been granted orphan drug designations in the U.S. and in the EU for some of our product candidates, however, there can be no guarantee that we will be able to maintain orphan status for these product candidates nor that we will receive orphan drug approval and prevent third parties from developing and commercializing products that are competitive to our product candidates.

To date, in addition to the orphan drug exclusivity described above for EXONDYS 51, we have been granted orphan drug designation by the FDA under the Orphan Drug Act for additional product candidates for the treatment of DMD and infectious diseases, including AVI-7537 for the treatment of Ebola virus and AVI-7288 for the treatment of the Marburg virus.

We also have been granted orphan medicinal product designations in the EU for two of our product candidates in DMD (including EXONDYS 51). Product candidates granted orphan status in Europe can be provided with up to ten years of marketing exclusivity, meaning that another application for marketing authorization of a later, similar medicinal product for the same therapeutic indication will generally not be approved in Europe during that time period. Although we may have product candidates that obtain orphan drug exclusivity in Europe, the orphan status and associated exclusivity period may be modified for several reasons, including a significant change to the orphan medicinal product designations or status criteria after-market authorization of the orphan product (*e.g.*, product profitability exceeds the criteria for orphan drug designation), problems with the production or supply of the orphan drug, or a competitor drug, although similar, is safer, more effective or otherwise clinically superior than the initial orphan drug.

As discussed above, we are not guaranteed to receive or maintain orphan status for our current or future product candidates, and if our product candidates that are granted orphan status were to lose their status as orphan drugs or the marketing exclusivity provided for them in the U.S. or the EU, our business and operations could be adversely affected. While orphan status for any of our products, if granted or maintained, would provide market exclusivity in the U.S. and the EU for the time periods specified above, we would not be able to exclude other companies from manufacturing and/or selling products using the same active ingredient for the same indication beyond the exclusivity period applicable to our product on the basis of orphan drug status. In addition, we cannot guarantee that another company will not receive approval to market a product candidate that is granted orphan drug status in the U.S. or the EU for the same indication as any of our product candidates for which we plan to file an NDA or MAA. If that were to happen, any pending NDA 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.

If we are unable to maintain orphan drug exclusivity for EXONDYS 51 in the U.S., we may face increased competition.

Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition affecting fewer than 200,000 people in the U.S. A company that first obtains FDA approval for a designated orphan drug for the specified rare disease or condition generally receives orphan drug marketing exclusivity for that drug for a period of seven years from the date of its approval. This orphan drug exclusivity prevents the approval of another drug containing the same active ingredient used for the same orphan indication, except in circumstances where, based on the FDA's determination, a subsequent drug is safer, more effective or makes a major contribution to patient care, or if the orphan drug manufacturer is unable to assure that a sufficient quantity of the orphan drug is available to meet the needs of patients with the rare disease or condition. Orphan drug exclusivity may also be lost if the FDA later determines that the initial request for designation was materially defective. EXONDYS 51 was granted orphan drug exclusivity for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping, which we expect will provide the drug with orphan drug marketing exclusivity in the U.S. until September 19, 2023, seven years from the date of its approval. However, such exclusivity may not effectively protect the product from competition if the FDA determines that a subsequent drug for the same indication is safer, more effective or makes a major contribution to patient care, or if we are unable to assure the FDA that sufficient quantities of EXONDYS 51 are available to meet patient demand. In addition, orphan drug exclusivity does not prevent the FDA from approving competing drugs for the same or similar indication containing a different active ingredient. If a subsequent drug is approved for marketing for the same or similar indication, we may face increased competition, and our revenues from the sale of EXONDYS 51 will be adversely affected.

We will incur significant liability if it is determined that we are promoting any "off-label" use of EXONDYS 51.

Physicians are permitted to prescribe drug products for uses that are not described in the product's labeling and that differ from those approved by applicable regulatory agencies. Off-label uses are common across medical specialties. Although the FDA and other regulatory agencies do not regulate a physician's choice of treatments, the FDA and other regulatory agencies do prohibit

advertising and promotion of off-label uses of approved drug products or promotion of an approved drug on information that is not in the final, FDA-approved label for a product and restrict communications on off-label use. Accordingly, we may not promote EXONDYS 51 in the U.S. for use in any indications other than for the treatment of DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. Additionally, we are not able to promote EXONDYS 51 based on any information excluded in the final FDA-approved label, including previously published clinical data. The FDA and other regulatory authorities actively enforce laws and regulations prohibiting promotion of a product for off-label uses and the promotion of products for which marketing approval has not been obtained. A company that is found to have improperly promoted its drug product will be subject to significant liability, including civil and administrative remedies as well as criminal sanctions.

Notwithstanding the regulatory restrictions on off-label promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non-misleading and non-promotional scientific exchange concerning their products. We intend to engage in medical education activities and communicate with healthcare providers in compliance with all applicable laws, regulatory guidance and industry best practices. Although we have established a compliance program and continue to enhance it to ensure that all such activities are performed in a legal and compliant manner, EXONDYS 51 is our first commercial product which could increase risk of non-compliance with our internal compliance policies and applicable rules and regulations, which could negatively impact our business.

Most of our product candidates are at an early stage of development and may never receive regulatory approval.

Other than EXONDYS 51, which the FDA approved for use in the U.S. in September 2016 and for which we filed an MAA in November 2016 with the EMA, our most advanced product candidates are exon 45 and 53 skipping products. We are in the process of conducting, starting or planning various EXONDYS 51 clinical studies including studies that are required to comply with regulatory NDA and/or MAA filing requirements as well as studies we need to conduct to comply with our post-marketing FDA requirements to verify and describe clinical benefit. The exon 53-skipping product candidate, which we are working on with the SKIP-NMD consortium, is currently in the clinic in EU. The Part I dose-titration portion of this Phase 1/2a study has been completed and Part II open label portion of the study is ongoing. We have also completed the dose titration portion and are conducting the open-label portion of a study for our exon 45-skipping product candidate. Additionally, we are enrolling patients in the U.S. and working towards initiating sites in the EU and Canada for a clinical trial using exon 45- and 53-skipping product candidates, which we refer to as the ESSENCE study. The remainder of our product candidates are in discovery or early stages of development. These product candidates will require significant further development, financial resources and personnel to develop into commercially viable products and obtain regulatory approval, if at all. Currently, our exon 45-skipping product candidate, the exon 53-skipping product candidate we are developing with the SKIP-NMD consortium, each for DMD, and radavirsen (formerly AVI-7100) for influenza are in active clinical development. Our other product candidates, including our anti-bacterials and AVI-7537 in Ebola and AVI-7288, are in discovery, pre-clinical development or inactive. Given the FDA approval of EXONDYS 51, we expect that much of our effort and many of our expenditures over the next several years will be devoted to clinical development and regulatory activities associated with EXONDYS 51 and other exon-skipping candidates as part of our larger follow-on exon strategy in DMD, our other disease candidates, our proprietary chemistry, and other potential therapeutic areas that provide long-term market opportunities. We may be delayed, restricted, or unable to further develop our active and other product candidates or successfully obtain approvals needed to market them. Although EXONDYS 51 was approved under accelerated approval by the FDA in the U.S., we may not be able to obtain an approval of EXONDYS 51 in the EU.

Our RNA-targeted antisense technologies have only been incorporated into one therapeutic commercial product and additional studies may not demonstrate safety or efficacy of our technologies in other product candidates.

Our RNA-targeted platform, utilizing proprietary PMO-based technology has only been incorporated into one therapeutic commercial product to date, EXONDYS 51, however, our confirmatory trials for EXONDYS 51 must verify and describe the clinical benefits in order for EXONDYS 51 to remain approved in the U.S. All of our product candidates to date use our PMO-based technology. Although we have conducted and are in the process of conducting clinical studies with EXONDYS 51, an exon 45-skipping product candidate and an exon 53-skipping product candidate and pre-clinical studies with our other product candidates that use our PMO-based antisense technology, additional studies may be needed to determine the safety and efficacy of our PMO-based antisense technology, including our novel PPMO technology. In addition, nonclinical models used to evaluate the activity and toxicity of product candidate compounds are not necessarily predictive of toxicity or efficacy of these compounds in the treatment of human disease. As such, there may be substantially different results observed in clinical trials from those observed in pre-clinical studies. Any failures or setbacks in developing or utilizing our PMO-based technologies, including adverse effects in humans, could have a detrimental impact on our product candidate pipeline and our ability to maintain and/or enter into new corporate collaborations regarding these technologies, which would negatively affect our business and financial condition.

Our pre-clinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, including those based on our PMO-based technologies, 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 studies that the product candidate is safe and effective in humans. Ongoing and future pre-clinical and clinical trials of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. For example, although the pre-clinical data for PPMO collected to date is promising, the additional data we collect, including in the clinic, may not be consistent with the pre-clinical data or show a safe benefit that warrants further development or pursuit of a regulatory approval for PPMO product candidates. 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. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, including for those that are based on our PMO-based technologies, then 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. For example, we cannot provide assurances that data from our EXONDYS 51 ongoing studies will be positive and consistent through the study periods or that the interpretation by regulators, such as the FDA or EMA, of the data we collect for our product candidates will be consistent with our interpretations.

If there are significant delays in obtaining or 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, which could impair our ability to generate sufficient revenue and have a successful business.

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., 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. 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 (“CMC”) 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 or MAA submissions for one of our product candidates, and may delay, reject or refuse to accept for review, or approve any NDA or MAA 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 we submit to the FDA. Even if we meet FDA requirements and an advisory committee votes to recommend approval of an NDA 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 DMD, that use new technologies and processes, such as antisense oligonucleotide therapies, and 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, 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. We cannot be sure that any of our product candidates will qualify for accelerated approval or any other 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. 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 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 patient muscle biopsies and dystrophin analyses), 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 result in a decision by the Company not to proceed with an NDA 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 the exon 53- and exon 45-skipping or other product candidates. Responding to requests from regulators and meeting requirements for clinical studies, 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 or MAA submissions.

Due to the above factors, among others, our product candidates could take a significantly longer time to gain regulatory approval than we expect, or may never gain regulatory approval, which would delay or eliminate any potential commercialization or product revenue for us and result in a material adverse effect on the Company that could involve changes, delays in or terminations of programs in our pipeline, delays or terminations of pre-clinical and clinical studies, and termination of contracts related to the development of our product candidates which can include significant termination costs, workforce reductions and limited ability to raise additional funds to execute company plans.

Even if we are able to comply with all regulatory requests and requirements, the delays resulting from satisfying such requests and requirements, the cost of compliance, or the effect of regulatory decisions (e.g., decisions limiting labeling and indications requested by us for a product candidate) may no longer make commercialization of a product candidate desirable for us from a business perspective, which could lead us to decide not to commercialize a product candidate.

Even after approval and commercialization of a product candidate, we remain subject to ongoing regulatory compliance and oversight to maintain our approval. Conducting our confirmatory studies could take years to complete, could yield negative or uninterpretable results or could result in an FDA determination that the studies do not provide the safety and efficacy requirements to maintain regulatory approval. If we are not able to maintain regulatory compliance, we may be subject to civil and criminal penalties or we may not be permitted to continue marketing our products, which could have a material adverse effect on our financial condition and harm our competitive position in the market place.

If we fail to comply with healthcare and other regulations, we could face substantial penalties and our business, operations and financial condition could be adversely affected.

As a manufacturer of pharmaceuticals, certain federal and state healthcare laws and regulations will apply to or affect our business. The regulations include:

- federal healthcare program anti-kickback laws, 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 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 physicians and teaching hospitals 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 health care 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.

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.

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. In particular, the Healthcare Reform Act includes a number of provisions aimed at strengthening the government's ability to pursue anti-kickback and false claims cases against pharmaceutical manufacturers and other healthcare entities, including substantially increased funding for healthcare fraud enforcement activities, enhanced investigative powers, and amendments to the False Claims Act that make it easier for the government and whistleblowers to pursue cases for alleged kickback and false claim violations. While it is too early to predict what effect these changes will have on our business, we anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of government investigations and enforcement actions. For example, federal enforcement agencies recently have shown interest in pharmaceutical companies' product and patient assistance programs, including manufacturer reimbursement support services and relationships with specialty pharmacies. Some of these investigations have resulted in significant civil and criminal settlements. Responding to a government investigation or enforcement action would be expensive and time-consuming, and could have a material adverse effect on our business and financial condition and growth prospects.

In connection with the commercial launch of EXONDYS 51, we have initiated our compliance program and are in the process of expanding our experienced compliance team that will continue to work towards developing a program based on industry best practices that is designed to ensure that our commercialization of EXONDYS 51 complies with all applicable laws, regulations and industry standards. As this program has not yet been tested and the requirements in this area are constantly evolving, we cannot be certain that our program will eliminate all areas of potential exposure. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. Any action against us for violation of these laws, even if we successfully defend against such action, could 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.

We rely on third parties to provide services in connection with our pre-clinical and clinical development programs. The inadequate performance by or loss of any of these service providers could affect our product candidate development.

Several third parties provide services in connection with our pre-clinical and clinical development programs, including in vitro and in vivo studies, assay and reagent development, immunohistochemistry, toxicology, pharmacokinetics, clinical assessments, data monitoring and management, statistical analysis and other outsourced activities. If these service providers do not adequately perform the services for which we have contracted or cease to continue operations and we are not able to quickly find a replacement provider or we lose information or items associated with our product candidates, our development programs may be delayed.

We are winding down our expired U.S. government contract, and thus further development of our Ebola and Marburg product candidates may be limited by our ability to obtain additional funding for these programs and by the intellectual property and other rights retained by the U.S. government.

We have historically relied on U.S. government contracts and awards to fund and support certain development programs, including our Ebola and Marburg programs. The July 2010 U.S. Department of Defense ("DoD") contract providing funds for our Marburg program expired in July 2014, and the Ebola portion of the contract was previously terminated by the DoD in 2012 for convenience of the DoD. We are currently involved in contract wind-down activities and may be subject to additional government audits prior to collecting final cost reimbursements and fees owed by the government. If we are not able to complete such audits or other government requirements successfully, then the government may withhold some or all of the currently outstanding amounts owed to us. We may explore and evaluate options to continue advancing the development of our Ebola and Marburg product

candidates, which may or may not include funding through U.S. government programs. As a result of government budgetary cuts, appropriations and sequestration, among other reasons, the viability of the government and its agencies as a partner for further development of our Ebola and Marburg programs, or other programs, is uncertain. The options for us to further develop product candidates that were previously developed under contracts with the U.S. government with third parties may be limited or difficult in certain respects given that, after termination or expiration of a U.S. government contract, the government has broad license rights in intellectual property developed under such contract. Therefore, the U.S. government may have the right to develop all or some parts of product candidates that we have developed under a U.S. government contract after such contract has terminated or expired.

We may not be able to successfully conduct clinical trials due to various process-related factors which could negatively impact our business plans.

The successful start and completion of any of our clinical trials within time frames consistent with our business plans is dependent on regulatory authorities and various factors, which include, but are not limited to, our ability to:

- recruit and retain employees, consultants or contractors with the required level of expertise;
- recruit and retain sufficient patients needed to conduct a clinical trial;
- enroll and retain participants, which is a function of many factors, including the size of the relevant population, the proximity of participants to clinical sites, activities of patient advocacy groups, the eligibility criteria for the trial, the existence of competing clinical trials, the availability of alternative or new treatments, side effects from the therapy, lack of efficacy, personal issues and ease of participation;
- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the contract research organizations ("CROs") involved in the clinical trial;
- negotiate contracts and other related documents with clinical trial parties and IRBs, 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 the Company to various risks;
- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;
- manage or resolve unforeseen adverse side effects during a clinical trial;
- conduct the clinical trials in a cost-effective manner, including managing foreign currency risk in clinical trials conducted in foreign jurisdictions and cost increases due to unforeseen or unexpected complications such as enrollment delays, or needing to outsource certain Company functions during the clinical trial; and
- execute clinical trial designs and protocols approved by regulatory authorities without deficiencies.

If we are not able to manage the clinical trial process successfully, our business plans could be delayed or be rendered unfeasible for us to execute within our planned or required time frames, or at all.

We have incurred operating losses since our inception and we may not achieve or sustain profitability.

We incurred an operating loss of $266.7 million for the twelve months ended December 31, 2016. Our accumulated deficit was $1.2 billion as of December 31, 2016. Although we launched EXONDYS 51 in the U.S. in September 2016, we believe that it will take us some time to attain profitability and positive cash flow from operations. Substantially all of our revenue to date has been derived from research and development contracts with the DoD, the last of which expired in July 2014. We have not yet generated significant revenues from product sales and have generally incurred expenses related to research and development of our technologies and product candidates, 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 our launch and commercialization of EXONDYS 51 in the U.S.;
- expand the global footprint of EXONDYS 51 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.

As a result, we expect to continue to incur significant operating losses for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when, or if, we will become profitable.

We will need additional funds to conduct our planned research, development, manufacturing and business development efforts. If we fail to attract and manage significant capital on acceptable terms or fail to enter into strategic relationships, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

We will likely require additional capital from time to time in the future in order to meet FDA post-marketing approval requirements and market and sell EXONDYS 51 as well as continue the development of product candidates in our pipeline, to expand our product portfolio and to continue or enhance our business development efforts. The actual amount of funds that we may need and the sufficiency of the capital we have or are able to raise will be determined by many factors, some of which are in our control and others that are beyond our control. The Company and our board of directors continue to assess optimization in the size and structure of the Company as well as in its strategic plans. For example, in March 2016, we announced a long-term plan to consolidate facilities within Massachusetts and closing our Corvallis, Oregon offices by end of year. Any failure on our part to strategically and successfully manage the funds we raise, with respect to factors within our control, could impact our ability to successfully commercialize EXONDYS 51 and continue developing our product candidates. Some of the factors partially or entirely outside of our control that could impact our ability to raise funds, as well as the sufficiency of funds the Company has to execute its business plans successfully, include the success of our research and development efforts, the status of our pre-clinical and clinical testing, costs and timing relating to securing regulatory approvals and obtaining patent rights, regulatory changes, competitive and technological developments in the market, regulatory decisions, and any commercialization expenses related to any product sales, marketing, manufacturing and distribution. An unforeseen change in these factors, or others, might increase our need for additional capital.

We would expect to seek additional financing from the sale and issuance of equity or equity-linked or debt securities, and we cannot predict that financing will be available when and as we need financing or that, if available, the financing terms will be commercially reasonable. If we are unable to obtain additional financing when and if we require it, or on commercially reasonable terms, this would have a material adverse effect on our business and results of operations.

If we are able to consummate such financings, the trading price of our common stock could be adversely affected and/or the terms of such financings may adversely affect the interests of our existing stockholders. To the extent we issue additional equity securities or convertible securities, our existing stockholders could experience substantial dilution in their economic and voting rights. Additional financing, if available, 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. We could also be required to seek funds through arrangements with collaborators or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates, or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

Further, we may also enter into relationships with pharmaceutical or biotechnology companies to perform research and development with respect to our technologies, research programs, conduct clinical trials or market our product candidates. Other than pre-clinical collaborations with academic or research institutions and government entities for the development of additional exon-

skipping product candidates for the treatment of DMD and clinical collaboration for a product candidate for the treatment of influenza, we currently do not have a strategic relationship with a third party to perform research or development using our technologies or assist us in funding the continued development and commercialization of any of our programs or product candidates. If we were to have such a strategic relationship, such third party may require us to issue equity to such third party, relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates, or to grant licenses on terms that may not be favorable to us.

Our indebtedness resulting from our credit and security agreement with MidCap Financial could adversely affect our financial condition or restrict our future operations.

On June 26, 2015, the Company entered into a credit and security agreement with MidCap Financial that provides a senior secured term loan of $20.0 million. This indebtedness could have important consequences, including:

- requiring the Company to maintain pledged cash in favor of MidCap Financial equal to but not less than the lesser of the outstanding term loans or $15.0 million;
- limiting our flexibility in planning for, or reacting to, changes in our business and our industry;
- placing us at a competitive disadvantage compared to our competitors who have less debt or competitors with comparable debt at more favorable interest rates;
- limiting our ability to borrow additional amounts for working capital, capital expenditures, research and development efforts, acquisitions, debt service requirements, execution of our business strategy and other purposes; and
- resulting in an acceleration of the maturity of such term loans upon the occurrence of a material adverse change or another default under the credit and security agreement.

Any of these factors could materially and adversely affect our business, financial condition and results of operations.

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

Our consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the U.S. 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 those related to revenue recognition, accrued expenses and assumptions in the valuation of stock-based compensation. 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, 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 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.

Our ability to use net operating loss carryforwards and other tax attributes to offset future taxable income may be limited as a result of future transactions involving our common stock.

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 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.” 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.

If we fail to retain our key personnel or are unable to attract and retain additional qualified personnel, our future growth and our ability to compete would 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 related 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 and motivate 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.

If we are unable to effectively manage our growth, execute our business strategy and implement compliance controls and systems, the trading price of our common stock could decline. Any failure to establish and maintain effective internal control over financial reporting could adversely affect investor confidence in our reported financial information.

We anticipate continued growth in our business operations due, in part, to the commercialization of EXONDYS 51. This future growth could create a strain on our organizational, administrative and operational infrastructure. 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 build the management and human resources and infrastructure necessary to support the growth of our business. The time and resources required to implement systems and infrastructure that may be needed to support our growth is uncertain, and failure to complete implementation in a timely and efficient manner could adversely affect our operations.

We may engage in future acquisitions or collaborations with other entities that increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We actively evaluate various strategic transactions on an ongoing basis, including licensing or acquiring complementary products, technologies or businesses. Potential acquisitions or collaborations with other entities may entail numerous risks, including increased operating expenses and cash requirements, assimilation of operations and products, retention of key employees, diversion of our management's attention and uncertainties in our ability to maintain key business relationships of the acquired entities. In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense.

Our success, competitive position and future revenue, if any, depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our technologies, product and product candidates, 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.

We currently hold various issued patents and exclusive rights to issued patents and own and have licenses to various patent applications, in each case in the U.S. as well as other countries. We anticipate filing additional patent applications both in the U.S. and in other countries. The patent process, however, is subject to numerous risks and uncertainties, and we can provide no assurance that we will be successful in obtaining and defending patents or in avoiding infringement of the rights of others. Even when our patent claims are allowed, the claims may not issue, or in the event of issuance, may not be sufficient to protect the technology owned by or licensed to us or our collaborators. Even if our patents and patent applications do provide our product, product candidates and platform technology with a basis for exclusivity, we and our collaborators may not be able to develop or commercialize such product and product candidates or platform technology due to patent positions held by one or more third parties.

We may not be able to obtain and maintain patent protection for our product or product candidates necessary to prevent competitors from commercializing competing product candidates. Our patent rights might be challenged, invalidated, circumvented or otherwise not provide any competitive advantage, and we might not be successful in challenging the patent rights of our competitors through litigation or administrative proceedings. For example, in July 2014, the Patent Trial and Appeal Board (the "PTAB") of the USPTO declared patent interferences between certain patents held by Sarepta (under license from the University of Western Australia, "UWA") and patent applications held by BioMarin (under license from Academisch Ziekenhuis Leiden, "AZL") related to exon 51 and exon 53 skipping therapies designed to treat DMD. In particular, the PTAB declared Interference No. 106,008, which identifies Sarepta's/UWA's U.S. Patent Nos. 7,807,816 and 7,960,541, both covering EXONDYS 51, as interfering with BioMarin's/AZL's U.S. Application No. 13/550,210. The PTAB also declared Interference No. 106,007, which identifies Sarepta's/UWA's U.S. Patent

No. 8,455,636, covering SRP-4053, as interfering with BioMarin's/AZL's U.S. Application No. 11/233,495. In September 2014, the PTAB declared a third patent interference relating to certain methods concerning the exon 51 skipping therapies that are the subject of Interference No. 106,008. In particular, the PTAB declared Interference No. 106,013, which identifies Sarepta's/UWA's U.S. Patent No. 8,486,907, which covers certain methods of using EXONDYS 51, as interfering with BioMarin's/AZL's U.S. Application No. 14/198,992. In addition, in a September 2014 Order in Interference No. 106,007, the PTAB authorized us to file a motion with the PTAB, which we filed in November 2014, requesting the declaration of a fourth interference relating to certain methods concerning the exon 53 skipping therapies that are the subject of Interference No. 106,007, including SRP-4053, and between Sarepta's/UWA's U.S. Patent No. 8,455,636 and BioMarin's/AZL's U.S. Application No. 14/248,279. In Interference No. 106,013, we received notice on September 29, 2015 that the PTAB had issued a decision that resulted in a judgment against Sarepta and an order for the cancellation of Sarepta's/UWA's U.S. Patent No. 8,486,907 that covers certain methods of using EXONDYS 51 thereby leaving open the possibility of BioMarin's/AZL's competing U.S. Application No. 14/198,992 to issue and, if so, potentially provide a basis for BioMarin to allege that EXONDYS 51 infringes a patent granting from this application. We filed a Request for Rehearing that requests the PTAB to continue this interference, and the PTAB denied our Request on December 29, 2015. We appealed this decision to the U.S. Court of Appeals for the Federal Circuit on March 28, 2016, and this appeal was docketed as Case Nos. 16-1937 (lead) & 16-2016 (consolidated). In Interference No. 106,007, the PTAB entered a judgment on the motions on April 29, 2016 to end this interference between U.S. Patent No. 8,455,636 held by Sarepta (under license from UWA) and U.S. Application No. 11/233,495 held by BioMarin (under license from AZL) related to exon 53 skipping therapies, including SRP-4053, designed to treat DMD. The PTAB ordered: (i) the final refusal of all claims of BioMarin's/AZL's U.S. Application No. 11/233,495, with the exception of claim 77; and (ii) cancellation of all claims in Sarepta's/UWA's U.S. Patent No. 8,455,636, in each case based on its decision of various motions. The PTAB denied our motion filed in November 2014 requesting the declaration of a fourth interference relating to certain methods concerning the exon 53 skipping therapies that are the subject of this Interference No. 106,007, including SRP-4053, and between Sarepta's U.S. Patent No. 8,455,636 and BioMarin's U.S. Application No. 14/248,279, thereby leaving open the possibility of BioMarin's/AZL's competing U.S. Application No. 14/248,279 to issue and, if so, potentially provide a basis for BioMarin to allege that our product candidate, SRP-4053, infringes a patent granting from this application. BioMarin appealed the decision from Interference No. 106,007 to the U.S. Court of Appeals for the Federal Circuit on June 28, 2016, and this appeal was docketed as Case No. 16-2262 and designated by the Court as a companion case to the exon 51 methods interference appeal (Case No. 16-1937). On September 20, 2016, the PTAB issued a judgment in Interference No. 106,008 against BioMarin/AZL and ordered the final refusal of all claims of AZL's application, U.S. Application No. 13/550,210. BioMarin/AZL appealed the decision to the U.S. Court of Appeals for the Federal Circuit on October 12, 2016, and this appeal was docketed as Case No. 17-1078 and designated by the Court as a companion case to the exon 51 methods interference appeal (Case No. 16-1937) and the exon 53 interference appeal (Case No. 16-2262). We cannot make any assurances about the outcome of the appeals of any of these three interferences, or any subsequent appeals or rehearings. Any adverse rulings on the appeal could come at any time and, if negative, could adversely affect our business and result in a decline in our stock price. If final resolution of the interference appeals are not in our favor, then the Sarepta/UWA patents involved in the interferences, any other Sarepta/UWA patents or applications also found to be interfering, and any other Sarepta/UWA patents or applications may be invalidated or subject to invalidation, and as a result, we may not have any patent-based exclusivity available for our product or product candidates, which may have a material negative impact on our business plans. In addition, if final resolution of the interference appeals are not in our favor, the USPTO may issue the BioMarin/AZL patent applications resulting in the grant of one or more patents that may provide a basis for BioMarin to allege that EXONDYS 51 and/or our product candidate, SRP-4053, infringe such patents. In addition, the interference appeals and any subsequent litigation may require significant financial resources that we may have planned to spend on other Company objectives, resulting in delays or other negative impacts on such other objectives. In addition, BioMarin may continue to evaluate other opportunities to challenge our intellectual property rights or seek to broaden their patent positions in an attempt to cover our product candidates in the U.S. and in other jurisdictions. We are also aware of certain pending and granted claims that are held by BioMarin in Japan, Europe and certain other countries that may provide the basis for BioMarin or other parties to assert that EXONDYS 51 infringes on such claims. Because we have not yet initiated an invalidation proceeding in these countries, the outcome and timing of any such proceeding cannot be predicted or determined as of the date of this report.

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. Additionally, jurisdictions other than the U.S. might have less restrictive patent laws than the U.S., giving foreign competitors the ability to exploit these laws to create, develop and market competing products. The USPTO and patent offices in other jurisdictions have often required that patent applications concerning 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.

On September 16, 2011, the Leahy-Smith America Invents Act (the "Leahy-Smith Act"), was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted, and may also affect patent litigation. The USPTO has issued regulations and procedures to govern administration of the Leahy-Smith Act, but many of the substantive changes to patent law associated with the Leahy-Smith Act have only recently become effective. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business.

However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition. For instance, a third party may petition the PTAB seeking to challenge the validity of some or all of the claims in any of our patents through an *Inter Partes Review* ("IPR") or other post-grant proceeding. Should the PTAB institute an IPR (or other) proceeding and decide that some or all of the claims in the challenged patent are invalid, such a decision, if upheld on appeal, could have a material adverse effect on our business and financial condition.

The full impact of several recent U.S. Supreme Court decisions relating to patent law is not yet known. For example, on March 20, 2012, in *Mayo Collaborative Services, DBA Mayo Medical Laboratories, et al. v. Prometheus Laboratories, Inc* ., the Court held that several claims drawn to measuring drug metabolite levels from patient samples and correlating them to drug doses were not patentable subject matter. The decision appears to impact diagnostics patents that merely apply a law of nature via a series of routine steps and it has created uncertainty around the ability to patent certain biomarker-related method claims. Additionally, on June 13, 2013, in *Association for Molecular Pathology v. Myriad Genetics, Inc.*, the Court held that claims to isolated genomic DNA are not patentable, but claims to complementary DNA molecules were held to be valid. The effect of the decision on patents for other isolated natural products is uncertain and, as with the Leahy-Smith Act, these decisions could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

Our business prospects will be impaired if third parties successfully assert that EXONDYS 51 or our product candidates or technologies infringe proprietary rights of such third parties.

Our competitors may make significant investments in competing technologies, and might have or obtain patents that limit, interfere with or eliminate our ability to make, use and sell EXONDYS 51 or our product candidates in important commercial markets.

If EXONDYS 51 or our product candidates or technologies 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;
- redesign EXONDYS 51, 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 substantially harm our potential earnings, financial condition and operations. BioMarin has rights to patent claims that, absent a license, may preclude us from commercializing EXONDYS 51 in several jurisdictions. BioMarin has rights to European Patent No. EP 1619249, for example. We opposed this patent in the Opposition Division of the European Patent Office ("EPO"), and the Opposition Division maintained certain claims of this patent relating to the treatment of DMD by skipping dystrophin exons 51 and 46, which may provide a basis to maintain that commercialization of EXONDYS 51 in a European country where BioMarin has a patent corresponding to EP 1619249 would infringe on such patent. Both we and BioMarin have appealed the Opposition Division decision, submitted briefs in support of our respective positions and have also submitted responses to each other's briefs. BioMarin filed arguments with the EPO in response to our previously filed briefs. The Opposition Division decision, if maintained at the appeals level, could have a substantial negative effect on our business and leaves open the possibility that BioMarin or other parties that have rights to such patent could assert that EXONDYS 51 infringes on such patent in a relevant European country. The timing and outcome of the appeal cannot be predicted or determined as of the date of this report. If as part of any appeal before the EPO we are unsuccessful in invalidating BioMarin's claims that were maintained by the Opposition Division or if claims previously invalidated by the Opposition Division are restored on appeal, our ability to commercialize both EXONDYS 51 and our therapeutic candidates could be materially impaired. Moreover, our ability to commercialize EXONDYS 51 in a European country where BioMarin has a patent related to EP 1619249 while the appeal process remains ongoing before the EPO Board of Appeals could be materially impaired. In addition, we are aware of various divisional applications relating to EP 1619249 that are being pursued by BioMarin, which are pending, granted and in some cases are proceeding to grant. One of these divisional applications that has granted claims exon 45 skipping antisense oligonucleotides (EP 2594641) and another claims exon 53 skipping antisense oligonucleotides (EP 2602322). We opposed EP 2594641 and EP 2602322 in the Opposition Division on September 29, 2016 and December 1, 2016, respectively. Any of these granted patents, should we be unsuccessful in invalidating them, or any related patents that grant, can also materially impair our ability to commercialize EXONDYS 51 or our therapeutic candidates, such as SRP-4045 and SRP-4053.

We are also aware of existing patent claims BioMarin is pursuing in the U.S., including those involved in the interferences declared by the USPTO in July 2014 and September 2014 and discussed in these risk factors, and others that it has or is pursuing in other countries, that where granted may provide the basis for BioMarin or other parties to assert that commercialization of EXONDYS 51 and certain other of our product candidates would infringe on such claims. Some of these existing patent claims have granted and may provide a basis for BioMarin to allege that EXONDYS 51 infringes such granted claims. These patent claims may materially impair our ability to commercialize EXONDYS 51.

The DMD patent landscape 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 EXONDYS 51 or our product candidates infringe on the intellectual property rights of such parties. Similarly, we may be able to assert that certain activities engaged in by these parties infringe on 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. We also cannot be certain that other third parties will not assert patent infringement in the future with respect to any of our development programs.

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. We are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development in areas related to antisense technology and other RNA technologies, or that are developing alternative approaches to or therapeutics for the disease indications on which we are focused. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with EXONDYS 51 or our product candidates. For example, we believe that companies including Alnylam Pharmaceuticals, Inc., Ionis Pharmaceuticals, Inc. (formerly Isis Pharmaceuticals, Inc.), Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Wave, Daiichi Sankyo and Nippon Shinyaku Co. Ltd. share a focus on RNA-targeted drug discovery and development. Competitors with respect to EXONDYS 51 or our product candidates include Nippon Shinyaku, Daiichi Sankyo, Wave and Shire plc; and other companies such as BioMarin (which acquired Prosensa), PTC and Summit have also been working on DMD programs. Additionally, several companies and institutions have entered into collaborations or other agreements for the development of product candidates, including mRNA, gene (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, Pfizer, Inc., Bristol-Myers Squibb, Biogen Idec, Inc., Ionis Pharmaceuticals, Inc., Alexion Pharmaceuticals, Inc., Sanofi, Eli Lilly, Alnylam, Moderna Therapeutics, Inc., Summit, Akashi, Catabasis, and Oxford University. 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 DMD, it further announced its intent to continue to explore the development of next generation oligonucleotides for the treatment of DMD.

If BioMarin or any of our competitors are successful in obtaining regulatory approval for any of their product candidates, it may limit our ability to gain or keep market share in the DMD space or other diseases targeted by our exon-skipping platform and product candidate pipeline.

It is possible that our competitors will succeed in developing technologies that limit the market size for EXONDYS 51 or our product candidates, impact the regulatory approval process for our product candidates that are more effective than our product candidates or that would render our technologies obsolete or noncompetitive. Our competitors may, among other things:

- develop safer or more effective products;
- implement more effective approaches to sales and marketing;
- develop less costly products;
- 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.

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, expanded access programs, the sale of EXONDYS 51 and future 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 limited general commercial liability insurance coverage for our clinical trials. We intend to expand our insurance coverage to include the sale of commercial products in connection with the FDA's approval of EXONDYS 51. 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.

Our operations involve the use of hazardous materials, and we must comply with environmental laws, which can be expensive, and may affect our business and operating results.

Our research and development activities involve the use of hazardous materials, including organic and inorganic solvents and reagents. Accordingly, we are subject to federal, state and local laws and regulations governing the use, storage, handling, manufacturing, exposure to and disposal of these hazardous materials. In addition, we are subject to environmental, health and workplace safety laws and regulations, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of bio-hazardous materials. 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.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cyber security 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 EXONDYS 51 patients, 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. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. Any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. 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, and employee matters. It is possible that we may not prevail in claims made against us in such disputes even after expending significant amounts of money and company resources in defending our positions in such lawsuits and 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.

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, has 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 twelve months, our stock has increased as much as 74% in a single day or decreased as much as 55% 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 EXONDYS 51 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 studies for potential product candidates;
- delays in entering or failing to enter into strategic relationships with respect to development and/or commercialization of EXONDYS 51 or our product candidates or entry into strategic relationships on terms that are not deemed to be favorable to our Company;
- 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 such as the stockholder lawsuits 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. In addition, 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.

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.

We expect our operating results to fluctuate in future periods, which may adversely affect our stock price.

Our operating results have fluctuated in the past, and we believe they will continue to do so in the future. Our operating results may fluctuate due to the variable nature of our revenue and research and development expenses. Likewise, our research and development expenses may experience fluctuations as a result of the timing and magnitude of expenditures incurred in support of our proprietary drug development programs. 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.

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 in the future. 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.

As of December 31, 2016, there were approximately 54.8 million shares of common stock outstanding and outstanding awards to purchase 5.7 million shares of common stock under various incentive stock plans. Additionally, as of December 31, 2016, there were approximately 3.0 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan, approximately 0.3 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan and approximately 1.1 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan. We may issue additional common stock and warrants from time to time to finance our operations. We may also issue additional shares to fund potential acquisitions or in connection with additional stock options or other equity awards granted to our employees, officers, directors and consultants under our Amended and Restated 2011 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. 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 options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

Item 1B. Unresolved Staff Comments.

None.

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, Massachusetts, Andover, Massachusetts and Corvallis, Oregon 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 02142	88,329	January – February 2021	Laboratory and office space	Corporate headquarters
100 Federal Street, Andover, MA	60,000	N/A – facility is owned	Manufacturing and office space	Primarily manufacturing space
4575 SW Research Way, Suite 200, Corvallis, OR 97333	53,000	December 2020	Laboratory and office space	Primarily lab space**
1749 SW Airport Avenue, Corvallis, OR 97333	36,150	N/A – facility is owned; land lease expires February 2042	Acquired with intention of providing future expansion space for the manufacture of potential products and components	The space is unoccupied.*

* In November 2011, the tenant, Perpetua Power Source Technologies, Inc. ("Perpetua"), agreed to lease approximately 25,000 square feet of the building until March 2017. In July 2016, Perpetua decided to discontinue to lease the space. As of the date of this report, the space remains unoccupied.

** As a result of the plan to close the Corvallis site, the second floor of this property has been vacated as of December 31, 2016.

Item 3. Legal Proceedings.

For material legal proceedings, please read *Note 16, Commitments and Contingencies - Litigation* to our consolidated financial statements included in this 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 symbol "SRPT." Prior to January 2, 2014, our Common Stock was quoted on The NASDAQ Global Market. The following table sets forth the high and low intraday sales prices as reported by The NASDAQ Global Select Market for each quarterly period in the two most recent years:

	High	Low
Year Ended December 31, 2016		
First Quarter	$ 38.80	$ 10.20
Second Quarter	$ 24.60	$ 8.00
Third Quarter	$ 63.73	$ 19.06
Fourth Quarter	$ 63.03	$ 26.66
Year Ended December 31, 2015		
First Quarter	$ 15.74	$ 11.33
Second Quarter	$ 33.16	$ 12.01
Third Quarter	$ 41.47	$ 28.19
Fourth Quarter	$ 41.97	$ 23.09

Holders

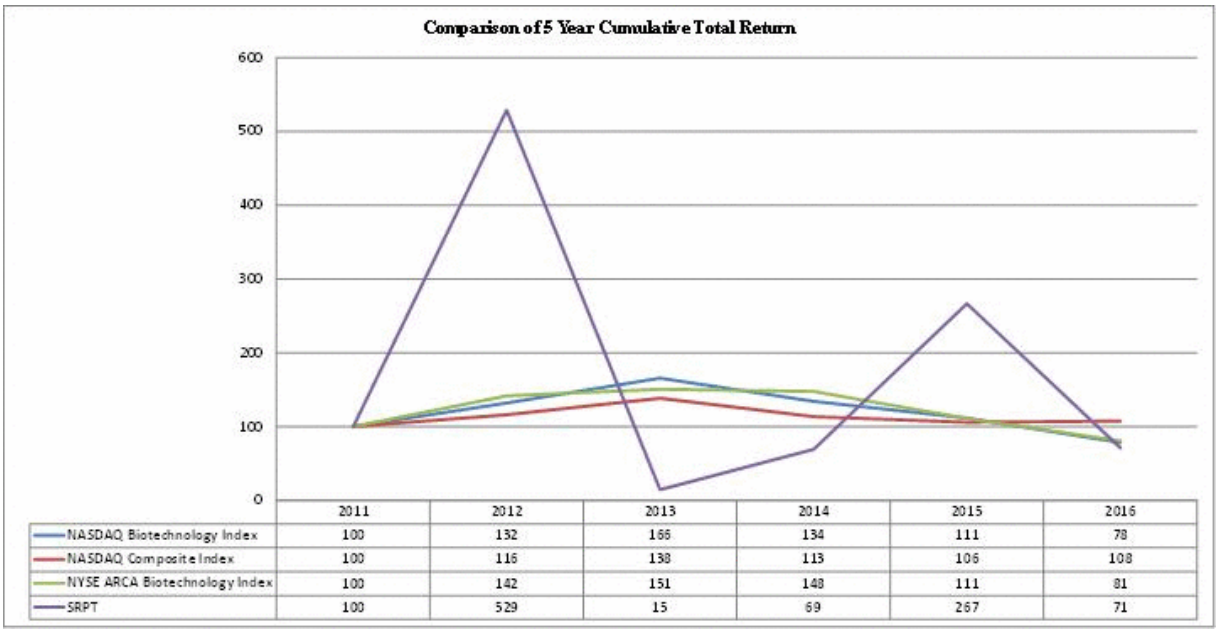
As of February 21, 2017, we had 230 stockholders of record of our common stock.

Dividends

We did not declare or pay cash dividends on our common stock in 2016, 2015 or 2014. 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 on December 31, 2011 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.



	2011	2012	2013	2014	2015	2016
NASDAQ Biotechnology Index	100	132	166	134	111	78
NASDAQ Composite Index	100	116	138	113	106	108
NYSE ARCA Biotechnology Index	100	142	151	148	111	81
SRPT	100	529	15	69	267	71

Recent Sales of Unregistered Securities.

None.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers.

None.

Item 6. Selected Financial Data.

The following selected financial data are derived from our consolidated financial statements and should be read in conjunction with, and is qualified in its entirety by, *Item 7, Management's Discussion and Analysis of Financial Condition and Results of Operations,* and *Item 8, Financial Statements and Supplementary Data.*

	For the Year Ended December 31,				
	2016	2015	2014	2013	2012
	(in thousands, except per share amounts)				
Operations data:					
Revenues	$ 5,421	$ 1,253	$ 9,757	$ 14,219	$ 37,329
Cost of sales	130	—	—	—	—
Research and development	188,272	146,394	94,231	72,909	52,402
Selling, general and administrative	83,749	75,043	49,315	31,594	14,630
Operating loss	(266,730)	(220,184)	(133,789)	(90,284)	(29,703)
Interest (expense) income and other, net	(535)	154	779	326	354
Loss on change in warrant valuation	—	—	(2,779)	(22,027)	(91,938)
Net loss	$ (267,265)	$ (220,030)	$ (135,789)	$ (111,985)	$ (121,287)
Net loss per share—basic and diluted	$ (5.49)	$ (5.20)	$ (3.39)	$ (3.31)	$ (5.14)
Balance sheet data:					
Cash and cash equivalents	$ 122,420	$ 80,304	$ 73,551	$ 256,965	$ 187,661
Working capital	298,054	162,249	210,929	234,840	115,022
Total assets	424,104	273,782	295,033	291,569	204,993
Stockholders' equity	336,691	190,347	247,653	247,192	123,679

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

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.

Overview

We are a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. Applying our proprietary, highly-differentiated and innovative platform technologies, we are able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action. We are primarily focused on rapidly advancing the development of our potentially disease-modifying pipeline of exon-skipping drug candidates targeting DMD. On September 19, 2016, the FDA granted accelerated approval for EXONDYS 51, indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 is studied in clinical trials under the name of eteplirsen and is marketed in the U.S. under the trademarked name of EXONDYS 51® (eteplirsen) Injection. We commenced shipments of EXONDYS 51 to customers at the end of the third quarter of 2016. Additionally, we submitted an MAA for eteplirsen to the EMA in November 2016 and the application was accepted in December 2016.

Our RNA-targeted technologies work at the most fundamental level of biology and potentially could have a meaningful impact across a broad range of human diseases and disorders. Our lead program focuses on the development of disease-modifying therapeutic candidates for DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. EXONDYS 51 is the first approved disease-modifying therapy for DMD in the U.S. and is also our first product candidate to receive marketing approval from the FDA. As of the date of this report, EXONDYS 51 has not been approved for sale or marketing by any regulatory agency or authority outside of the U.S.

The basis of our novel RNA-targeted therapeutics is the PMO. Our next generation PMO-based chemistries include PPMO, PMO-X® and PMO*plus*®. PMO-based compounds are highly resistant to degradation by enzymes, potentially enabling robust and sustained biological activity. In contrast to other RNA-targeted therapeutics, which are usually designed to down-regulate protein expression, our technologies are designed to selectively up-regulate or down-regulate protein expression, and more importantly, create novel proteins. PMO-based compounds have demonstrated inhibition of mRNA translation and alteration of pre-mRNA splicing. PMO-based compounds have the potential to reduce off-target effects, such as the immune stimulation often observed with ribose-based RNA technologies. We believe that our highly differentiated, novel, proprietary and innovative RNA-targeted PMO-based platforms may represent a significant improvement over other RNA-targeted technologies. In addition, PMO-based compounds are highly adaptable molecules: with minor structural modifications, they can potentially be rapidly designed to target specific tissues, genetic sequences, or pathogens, and therefore, we believe they could potentially be applied to treat a broad spectrum of diseases.

PPMO, our next generation chemistry, features covalent attachment of a cell-penetrating peptide to a PMO with the goal of enhanced cellular delivery into the cytosol and the nucleus. Based on our pre-clinical research, we believe our proprietary class of PPMO compounds demonstrates enhanced efficacy as compared to PMO and has the potential to provide improved delivery and dystrophin production in vivo, favorable tolerability in non-human primates, and a more durable response, which may support less frequent dosing and can potentially be tailored to target any organ. We plan on beginning IND-enabling GLP toxicology studies during early 2017 and we are targeting opening an IND before year-end in 2017 for DMD exon 51.

We are in the process of conducting, starting, or planning several studies in the U.S. and Europe for EXONDYS 51 and other product candidates designed to skip exons 45 and 53. These are comprised of:

(i) studies we are currently conducting to further evaluate EXONDYS 51, including an open label extension of our Phase 2b study, the PROMOVI study (an open label study on ambulatory patients with a concurrent untreated control arm), a study on participants with advanced stage DMD and a study on participants with early stage DMD, each of which will allow for patients to transition to commercial drug after meeting certain criteria;

(ii) EXONDYS 51 studies we are planning to initiate to comply with U.S. and/or EU regulatory requirements for IND applications and MAAs, respectively (e.g. a Phase 4 study on participants between the ages of six months and four years in connection with our PIP in the EU, and two additional Phase 1 studies);

(iii) studies we are planning to fulfill for our post-marketing FDA requirements/commitments for EXONDYS 51 and SRP-4045 and SRP-4053, our product candidates designed to skip exons 45 and 53, respectively;

(iv) a dose-ranging study that we completed for our product candidate designed to skip exon 45 that has transitioned into an open-label study;
(v) a two-part randomized, double-blind, placebo-controlled, dose titration safety, tolerability and pharmacokinetics study for a product candidate designed to skip exon 53 for which we have completed Part I and have now transitioned into Part II, an open label efficacy and safety study; and
(vi) ESSENCE, a placebo-controlled study with product candidates designed to skip exons 45 and 53 which has begun enrolling patients in the U.S. and for which we plan to have sites in the EU and Canada.

We believe we have developed proprietary state-of-the-art manufacturing and techniques that allow synthesis and purification of our product candidates to support both clinical development as well as commercialization. We have entered into certain manufacturing and supply arrangements with third-party suppliers which will in part utilize these techniques to support production of certain of our product candidates and their components. We currently do not have any of our own internal manufacturing capabilities to produce our product and product candidates for commercial and/or clinical use.

As of December 31, 2016, we had approximately $329.3 million of cash, cash equivalents and investments, consisting of $122.4 million of cash and cash equivalents, $195.4 million of short-term investments and $11.5 million of restricted cash and investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve months.

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 programs and the complex regulatory environment in which we operate. We may never achieve significant revenue or profitable operations.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations are 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 accounting principles generally accepted in the U.S. 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:

- revenue recognition;
- Inventory;
- research and development expense;
- stock-based compensation; and
- income tax.

Revenue Recognition

Revenues are recognized when persuasive evidence of an arrangement exists, delivery has occurred and title of the product and associated risk of loss has passed to the customer, the price is fixed or determinable and collection from the customer has been reasonably assured.

Product revenue, net

We are currently approved to sell EXONDYS 51 in the U.S. We distribute our product principally through a limited number of specialty distributor and specialty pharmacies (collectively, “Customers”). Title and risk of loss transfers upon delivery of EXONDYS 51 to Customers’ facilities. Our Customers subsequently resell our products to patients and health care providers. We provide no right of return to Customers in the U.S. except in cases of shipping error or product defect. Product revenues are recorded net of estimated rebates, Public Health Service (“PHS”) chargebacks and co-pay assistance.

We establish reserves for Medicaid rebates, PHS chargebacks and co-pay assistance. These reserves are based on estimates of the amounts earned or to be claimed on the related sales. Our estimates take into consideration current contractual and statutory requirements. If actual results vary, we may need to adjust these estimates, which could have an effect on earnings in the period of the adjustment. In addition to the discounts and rebates described above and classified as a reduction of revenue, we also maintain certain customer service contracts with Customers in the distribution channel that provide us with inventory management, data and distribution services, which are generally accounted for as a reduction of revenue. To the extent we can demonstrate a separable benefit and fair value for these services, we classify these payments within selling, general and administrative expenses.

Revenue from research contracts and other grants

Our contracts with the U.S. government are generally cost plus contracts providing for reimbursed costs which include overhead, general and administrative costs and a target fee. We recognize revenue from U.S. government research contracts during the period in which the related expenses are incurred and present such revenues and related expenses on a gross basis in the consolidated financial statements. Our government contracts are subject to government audits, which may result in catch-up adjustments. As of December 31, 2014, we had completed all development activities of our contracts with the U.S. government. The majority of the revenue under our U.S. government contracts was recognized as of December 31, 2015 and only revenue for contract finalization, if any, is expected in the future.

Deferred revenue

If a technology, right, product or service is separate and independent of our performance under other elements of an arrangement, we defer recognition of non-refundable up-front fees if we have continuing performance obligations when the technology, right, product or service conveyed in conjunction with the non-refundable fee has no utility to the licensee. In addition, if we have continuing involvement through research and development services that are required because of our know-how or because the services can only be performed by us, such up-front fees are deferred and recognized over the period of continuing involvement. As of December 31, 2016, we had deferred revenue of $3.3 million, which represents up-front fees we may recognize as revenue upon settlement of certain obligations.

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 revenue recognition.

Inventory

Inventories are stated at the lower of cost or estimated net realizable value, on a first-in, first-out basis. We capitalize inventory costs associated with our products upon regulatory approval. Prior to regulatory approval, we expense costs incurred to manufacture our drug products that could potentially be available to support the commercial launch of our products. Beginning in the third quarter of 2016 following the FDA approval of EXONDYS 51, we began to capitalize inventory costs associated with EXONDYS 51 when it was determined that the inventory had a probable future economic benefit. Additionally, we periodically analyze our inventory levels, and write down inventory that has become obsolete, inventory that has a cost basis in excess of its estimated net realizable value and inventory in excess of expected sales requirements as cost of sales. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required. 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 inventory.

Research and Development Expenses

All research and development expenses are expensed as incurred and are comprised of:

- employee-related expenses, including salaries, bonuses, benefits, travel and stock-based compensation expense;
- fees and expenses incurred under agreements with contract research organizations, investigative sites and other entities in connection with the conduct of clinical trials and preclinical studies and related services, such as data management, laboratory and biostatistics services;
- the cost of acquiring, developing and manufacturing APIs for product candidates that have not received regulatory approval;
- fees and costs related to regulatory filings and activities;
- 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;
- facilities, depreciation and other expenses, including rent, utilities, maintenance of facilities, insurance and other supplies.

When third-party service providers' billing terms do not coincide with our period-end, we are required to make estimates of our obligations to those third parties, including clinical trial and pharmaceutical development costs, contractual services costs and costs for supply of our drug candidates, incurred in a given accounting period and record accruals at the end of the period. We base our estimates on our knowledge of the research and development programs, services performed for the period and past history, where applicable. 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 research and development expenses.

Stock Compensation Expense

We use the fair value method to determine stock-based compensation expense. To determine the fair value of stock-based awards on the date of grant, we use the Black-Scholes-Merton option-pricing model. The Black-Scholes-Merton option-pricing model requires the use of subjective assumptions which include the award's expected term and the price volatility of the underlying stock. We recognize the fair value of the portion of the awards expected to vest as expense over the requisite vesting periods on a straight-line basis for the entire award. Stock awards granted to employees vest over a four-year period and have a ten-year term. Forfeitures are estimated at the time of grant and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates.

The assumptions used in calculating the fair value of stock-based compensation expense represent management's best estimates, but these estimates involve inherent uncertainties and the application of management judgment. As a result, if factors change and we use different assumptions, our stock-based compensation expense could be materially different in the future. Please read *Note 12, Stock-Based Compensation* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for a further discussion of stock-based compensation.

Income Tax

We follow the asset and liability method of accounting for income taxes. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the consolidated financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. It is our intention to reinvest the earnings of our non-U.S. subsidiaries in those operations and not to repatriate the earnings to the U.S. Accordingly, we do 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. To date, we have not had any earnings in our non-U.S. subsidiaries.

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered and settled. A valuation allowance is recorded to reduce the net deferred tax asset to zero because it is more likely than not that the net deferred tax asset will not be realized. We recognize the effect of income tax positions only if those positions are more likely than not to be sustained upon an examination. Please read *Note 14, Income Taxes* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for a further discussion of income tax.

Results of Operations for the years ended December 31, 2016, 2015 and 2014

The following table sets forth selected consolidated statements of operations data for each of the periods indicated:

	For the Year Ended December 31			
	2016	2015	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$ 5,421	$ —	$ 5,421	NA
Revenue from research contracts and other grants	—	1,253	(1,253)	(100)%
Total revenues	5,421	1,253	4,168	333%
Cost and expenses:				
Cost of sales	130	—	130	NA
Research and development	188,272	146,394	41,878	29%
Selling, general and administrative	83,749	75,043	8,706	12%
Total cost and expenses	272,151	221,437	50,714	23%
Operating loss	(266,730)	(220,184)	(46,546)	21%
Other (loss) income:				
Interest (expense) income and other, net	(535)	154	(689)	(447)%
Net loss	$ (267,265)	$ (220,030)	$ (47,235)	21%
Net loss per share — basic and diluted	$ (5.49)	$ (5.20)	$ (0.29)	6%

	For the Year Ended December 31			
	2015	2014	Change	Change
	(in thousands, except per share amounts)		$	%
Revenue from research contracts and other grants	$ 1,253	$ 9,757	$ (8,504)	(87)%
Operating expenses:				
Research and development	146,394	94,231	52,163	55%
General and administrative	75,043	49,315	25,728	52%
Total operating expenses	221,437	143,546	77,891	54%
Operating loss	(220,184)	(133,789)	(86,395)	65%
Other income (loss):				
Interest income and other, net	154	779	(625)	(80)%
Loss on change in warrant valuation	—	(2,779)	2,779	(100)%
Net loss	$ (220,030)	$ (135,789)	$ (84,241)	62%
Net loss per share — basic and diluted	$ (5.20)	$ (3.39)	$ (1.81)	53%

Revenues

We record product revenues net of applicable discounts and allowances which include Medicaid rebates, PHS chargebacks and co-pay. Reserves established for these discounts and allowances are classified as reductions of accounts receivable (if the amount is payable to our customer) or a liability (if the amount is payable to a party other than our customer). These reserves are based on estimates of the amounts earned or to be claimed on the related sales. Our estimates take into consideration current contractual and statutory requirements. Actual amounts may ultimately differ from our estimates. If actual results are different from our estimates, we adjust these estimates, which will have an effect on earnings in the period of adjustment.

We recognize revenue from U.S. government research contracts and grants during the period in which the related expenses are incurred and present such revenues and related expenses on a gross basis in the consolidated financial statements. Our government contracts are subject to government audits, which may result in catch-up adjustments. As of December 31, 2014, we had completed all development activities of our contracts with the U.S. government. The majority of the revenue under our U.S. government contracts was recognized as of December 31, 2015 and only revenue for contract finalization, if any, is expected in the future.

Product revenues for EXONDYS 51 were $5.4 million in 2016. These revenues are related to sales of EXONDYS 51 following the September 2016 commercial launch of EXONDYS 51 in the U.S. Revenue from research contracts and other grants decreased by $1.3 million in 2016, or 100% compared to 2015, as all of the work related to government grants was completed prior to 2016. Only revenue from contract finalization, if any, is expected to be recognized in future periods.

Revenues for 2015 decreased by $8.5 million, or 87%, compared to 2014. For the year ended December 31, 2015, we recognized $1.3 million from contract finalization of the Ebola portion of the July 2010 DoD contract for the advanced development of our hemorrhagic virus therapeutic candidates against the Ebola and Marburg viruses, which was collected from the U.S. government in 2016. For the year ended December 31, 2014, we recognized $9.8 million under various U.S. government contracts.

Cost of Sales

Our cost of sales relates to sales of EXONDYS 51 following its commercial launch in the U.S. Prior to receiving regulatory approval for EXONDYS 51 from the FDA in September 2016, we expensed such manufacturing and material costs as research and development expenses. For EXONDYS 51 sold in 2016, the majority of related manufacturing costs incurred had previously been expensed as research and development expenses, as such costs were incurred prior to the FDA approval of EXONDYS 51. Therefore, the cost of sales presented in the consolidated statements of operations and comprehensive loss only included the cost of packaging and labeling for commercial sales as well as amortization of an in-licensed right. If product related costs had not previously been expensed as research and development expenses prior to receiving FDA approval, the cost to produce the EXONDYS 51 sold would have been approximately $0.5 million.

Research and Development Expenses

Research and development expenses consist of costs associated with research activities as well as costs associated with our product development efforts, conducting preclinical studies, 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 which 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 clinical programs include salaries, stock-based compensation and allocation of our facility costs.

Future research and development expenses may increase as our internal projects, such as those for our DMD product candidates, enter or proceed through later stage clinical development. We are currently conducting various clinical trials for EXONDYS 51, including a confirmatory trial in the U.S. We completed Part I and have started conducting Part II of a Phase 1/2a clinical trial for an exon 53-skipping product candidate in the EU. We have completed the dose titration portion and are conducting the open-label portion of a study for our exon 45-skipping product candidate. We have initiated a placebo-controlled study with product candidates designed to skip exons 45 and 53 in the U.S. and the EU. The remainder of our research and development programs are in various stages of research and pre-clinical development. However, our research and development efforts may not result in any approved products. Product candidates that appear promising at early stages of development may not reach the market for a variety of reasons. Similarly, any of our product candidates may be found to be unsafe or ineffective during clinical trials, may have clinical trials that take longer to complete than anticipated, may fail to receive necessary regulatory approvals, or may prove impracticable to manufacture in commercial quantities at reasonable cost and with acceptable quality.

As a result of these uncertainties and risks inherent in the drug development process, we cannot determine the duration or completion costs of current or future clinical stages of any of our product candidates. Similarly, we cannot determine when, if, or to what extent we may generate revenue from the commercialization of any product candidate. The time frame for development of any product candidate, associated development costs and the probability of regulatory and commercial success vary widely.

Our research and development programs span various disease targets. The lengthy process of securing FDA approvals for new drugs requires substantial resources. Accordingly, we cannot currently estimate, with any degree of certainty, the amount of time or money that we will be required to expend in the future on our product candidates prior to their regulatory approval, if such approval is ever granted.

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			
	2016	2015	Change	Change
	(in thousands)		$	%
EXONDYS 51 (exon 51)	$ 65,454	$ 72,147	$ (6,693)	(9)%
Exon 45	9,562	6,649	2,913	44%
Exon 53	11,847	5,583	6,264	112%
Other projects	1,248	2,178	(930)	(43)%
Up-front and milestone payments	48,035	165	47,870	29,012%
Internal research and development expenses	52,126	59,672	(7,546)	(13)%
Total research and development expenses	$ 188,272	$ 146,394	$ 41,878	29%

	For the Year Ended December 31			
	2015	2014	Change	Change
	(in thousands)		$	%
EXONDYS 51 (exon 51)	$ 72,147	$ 29,395	$ 42,752	145%
Exon 45	6,649	4,343	2,306	53%
Exon 53	5,583	8,013	(2,430)	(30)%
Other projects	2,178	4,196	(2,018)	(48)%
Milestone payments	165	—	165	NA
Internal research and development expenses	59,672	48,284	11,388	24%
Total research and development expenses	$ 146,394	$ 94,231	$ 52,163	55%

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

	For the Year Ended December 31			
	2016	2015	Change	Change
	(in thousands)		$	%
Clinical and manufacturing expenses	$ 82,077	$ 80,977	$ 1,100	1%
Up-front and milestone payments	48,035	165	47,870	29012%
Compensation and other personnel expenses	21,322	25,746	(4,424)	-17%
Stock-based compensation	9,499	10,403	(904)	-9%
Facility-related expenses	8,095	9,919	(1,824)	-18%
Professional services	7,537	8,329	(792)	-10%
Preclinical expenses	3,415	3,948	(533)	(14)%
Restructuring expenses	2,013	—	2,013	NA
Research and other	6,279	6,907	(628)	(9)%
Total research and development expenses	$ 188,272	$ 146,394	$ 41,878	29%

	For the Year Ended December 31			
	2015	2014	Change	Change
	(in thousands)		$	%
Clinical and manufacturing expenses	$ 80,977	$ 39,505	$ 41,472	105%
Compensation and other personnel expenses	25,746	20,234	5,512	27%
Stock-based compensation	10,403	8,269	2,134	26%
Facility-related expenses	9,919	7,792	2,127	27%
Professional services	8,329	7,689	640	8%
Preclinical expenses	3,948	2,758	1,190	43%
Milestone payments	165	—	165	NA
Research and other	6,907	7,984	(1,077)	(13)%
Total research and development expenses	$ 146,394	$ 94,231	$ 52,163	55%

Research and development expenses for 2016 increased by $41.9 million, or 29%, compared to 2015. The increase was primarily driven by increases of $47.5 million in up-front and milestone payments related to the Collaboration Agreement with Summit and the Amended and Restated UWA License Agreement and its First Amendment with UWA, $2.0 million in restructuring expenses, and $1.1 million in clinical and manufacturing expenses due to increased patient enrollment in our ongoing clinical trials, partially offset by lower manufacturing expenses because of the capitalization of inventory upon the approval of EXONDYS 51 by the

FDA. The increases were partially offset by decreases of $4.4 million in compensation and other personnel expenses, $1.8 million in facility-related expenses and $0.9 million in stock-based compensation primarily driven by decreases in headcount.

Research and development expenses for 2015 increased by $52.2 million, or 55%, compared to 2014. The increase was primarily due to increases of $41.5 million in clinical and manufacturing expenses, driven by increased enrollment in our ongoing clinical trials and timing of manufacturing activities (including raw material purchases) as well as expense incurred in connection with an amendment to a supply agreement, $5.5 million in compensation and other personnel expenses primarily driven by increases in headcount, $2.1 million in stock-based compensation and $2.1 million from facility-related expenses primarily driven by corporate growth.

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 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			
	2016	2015	Change $	Change %
	(in thousands)			
Compensation and other personnel expenses	$ 29,807	$ 17,513	$ 12,294	70%
Stock-based compensation	20,463	12,329	8,134	66%
Professional services	19,372	25,884	(6,512)	(25)%
Facility-related expenses	4,669	2,838	1,831	65%
Restructuring expenses	2,548	—	2,548	NA
Former CEO severance expense	—	9,182	(9,182)	NA
Other	6,886	7,297	(411)	(6)%
Total selling, general and administrative expenses	$ 83,745	$ 75,043	$ 8,702	12%

	For the Year Ended December 31			
	2015	2014	Change $	Change %
	(in thousands)			
Professional services	$ 25,884	$ 16,363	$ 9,521	58%
Compensation and other personnel expenses	17,513	12,454	5,059	41%
Stock-based compensation	12,329	11,921	408	3%
Former CEO severance expense	9,182	—	9,182	NA
Facility-related expenses	2,838	2,616	222	8%
Other	7,297	5,961	1,336	22%
Total general and administrative expenses	$ 75,043	$ 49,315	$ 25,728	52%

Selling, general and administrative expenses for 2016 increased by $8.7 million, or 12%, compared to 2015. The increase was primarily due to increases of $12.3 million in compensation and other personnel expenses, $8.1 million in stock-based compensation and $1.8 million in facility-related expenses. These increases were primarily driven by increases in commercial headcount. Additionally, we also incurred $2.5 million in restructuring expenses in 2016. The increases were partially offset by decreases of $9.2 million in severance expense related to the resignation of our former CEO in March 2015 and $6.4 million in professional services primarily due to lower litigation activities.

General and administrative expenses for 2015 increased by $25.7 million, or 52%, compared to 2014. The increase was primarily due to $9.5 million in professional services driven by increased legal fees and preparation for the product launch, $9.2 million in severance expense, including stock-based compensation, as a result of the resignation of our former CEO, and $5.1 million in compensation and other personnel expenses primarily driven by increases in headcount.

Interest (expense) income and other, net

Interest (expense) income and other, net, primarily consists of interest income on our cash, cash equivalents and investments, interest expense and rental income and loss. Our cash equivalents and investments consist of commercial paper, government and government agency debt securities, money market investments and certificates of deposit. Interest expense includes interest accrued on our promissory note related to the Andover, Massachusetts facility, our senior secured term loan and our mortgage loan related to our Corvallis, Oregon property. Rental income and loss is from leasing excess space in some of our facilities.

Interest expense and other, net for 2016 was $0.5 million compared to interest income and other, net of $0.2 million in 2015. The unfavorable change was primarily due to interest expense incurred in connection with the $20.0 million senior secured term loan.

Interest income and other, net for 2015 decreased by $0.6 million compared to 2014. The decrease was primarily driven by an increase in interest expense incurred in connection with the $20.0 million senior secured term loan.

Loss on Change in Warrant Valuation

Warrants issued in connection with our January and August 2009 financings were classified as liabilities as opposed to equity due to their settlement terms. These warrants were classified as non-cash liabilities. The fair value of these warrants was recorded on our consolidated balance sheets at the date of issuance. The warrants were marked to market at each financial reporting period, with changes in the fair value recorded as "Gain (loss) on change in warrant valuation" in our consolidated statements of operations and comprehensive loss. The fair value of the warrants was determined using the Black-Scholes-Merton option-pricing model, which required the use of significant judgment and estimates related to the inputs used in the model. All warrants issued in January and August 2009 were exercised or expired during 2014.

Liquidity and Capital Resources

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

	As of December 31, 2016	As of December 31, 2015	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 122,420	$ 80,304	$ 42,116	52%
Short-term investments	195,425	112,187	83,238	74%
Restricted cash and investments	11,479	11,478	1	0%
Total cash, cash equivalents and investments	$ 329,324	$ 203,969	$ 125,355	61%
Borrowings:				
Long-term debt	$ 16,150	$ 20,905	$ (4,755)	(23)%
Notes payable	—	2,493	(2,493)	(100)%
Total borrowings	$ 16,150	$ 23,398	$ (7,248)	(31)%
Working capital				
Current assets	$ 373,476	$ 224,543	$ 148,933	66%
Current liabilities	75,422	62,294	13,128	21%
Total working capital	$ 298,054	$ 162,249	$ 135,805	84%

For the year ended December 31, 2016, our principal source of liquidity was from equity financings. For the year ended December 31, 2015, our principal source of liquidity was from an equity and a debt financing. Our principal uses of cash are research and development expenses, general and administrative expenses, investments, capital expenditures and other working capital requirements.

Our future expenditures and capital requirements may be substantial and will depend on many factors, including but not limited to the following:

- our ability to generate revenues from sales of EXONDYS 51 and potential future products;
- the timing and costs of building out our manufacturing capabilities;
- the timing of advanced payments related to our future inventory commitments;

- the timing and costs associated with our clinical trials and preclinical studies;
- the attainment of milestones and our obligations to make milestone payments to Summit, UWA and other institutions; and
- the costs of filing, prosecuting, defending and enforcing patent claims and our other intellectual property rights.

Our cash requirements are expected to continue to increase as we advance our research, development and commercialization programs and we expect to seek additional financing primarily from, but not limited to, the sale and issuance of equity, debt securities or the licensing or sale of our technologies. 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 results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

Cash Flows

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

	For the Year Ended December 31			
	2016	2015	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ (245,820)	$ (149,465)	$ (96,355)	64%
Investing activities	(90,193)	8,410	(98,603)	(1,172)%
Financing activities	378,129	147,808	230,321	156%
Increase in cash and cash equivalents	$ 42,116	$ 6,753	$ 35,363	524%

	For the Year Ended December 31			
	2015	2014	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ (149,465)	$ (128,539)	$ (20,926)	16%
Investing activities	8,410	(159,030)	167,440	(105)%
Financing activities	147,808	104,155	43,653	42%
Increase (decrease) in cash and cash equivalents	$ 6,753	$ (183,414)	$ 190,167	(104)%

Operating Activities.

Cash used in operating activities for 2016 increased by $96.4 million compared to 2015. The increase was primarily due to an increase of $47.2 million in net loss driven by increases in research and development and selling, general and administrative expenses slightly offset by product revenue, an unfavorable change of $47.8 million in operating assets and liabilities due to the timing of certain activities and a decrease in non-cash adjustment of $1.4 million.

Cash used in operating activities for 2015 increased by $20.9 million compared to 2014. The increase was primarily due to an increase of $84.2 million in net loss driven by increases in research and development and general and administrative expenses partially offset by a favorable change of $54.1 million in operating assets and liabilities due to the timing of certain activities and an increase in non-cash adjustments of $9.2 million.

Investing Activities.

Cash used in investing activities for 2016 was $90.2 million compared to $8.4 million provided by investing activities in 2015. The change was primarily due to a decrease of $73.8 million in proceeds from the maturities and sales of available-for-sale securities, increases of $33.4 million in purchases of available-for-sale securities and $1.9 million in purchases of property and equipment. These were offset by a decrease of $10.7 million due to the purchase of a restricted investment.

Cash provided by investing activities was $8.4 million for 2015 while cash used in investing activities was $159.0 million for 2014. The change was primarily due to an increase of $51.0 million in proceeds from the maturity of available-for-sale securities and decreases of $112.4 million from the purchase of available-for-sale securities and $22.0 million from the purchase of property and equipment partially offset by the purchase of a restricted investment of $10.7 million. Additionally, for the year ended December 31, 2014, $7.3 million of restricted investments matured.

Financing Activities.

Cash provided by financing activities in 2016 increased by $230.3 million compared to 2015. In June and September 2016, we sold approximately 5.8 million and 2.1 million shares of common stock, generating net proceeds of $327.4 million and $37.3 million, respectively, $244.9 million higher than the prior year's equity offering. Additionally, net proceeds from stock option exercises and the employee stock purchase program was $10.0 million higher than 2015. The increases were partially offset by $5.0 million of repayments of long-term debt and notes payable and a decrease of $19.6 million in proceeds from our senior long-term secured loan.

Cash provided by financing activities in 2015 increased by $43.7 million compared to 2014. In October 2015, we sold approximately 3.3 million shares of common stock at an offering price of $39.00 per share. After deducting the underwriting discounts and offering related transaction costs, we received aggregate net proceeds of approximately $119.9 million, $25.4 million higher than the prior year's equity offering. Additionally, we received net proceeds of $19.6 million from the senior secured term loan and an incremental $9.0 million from option exercises. The increases were partially offset by a $2.5 million repayment of the promissory note related to our Andover, Massachusetts facility and a decrease of $7.8 million from warrant exercises that occurred in 2014.

Off-Balance Sheet Arrangements

During the periods presented, we did not have any relationships with unconsolidated entities or financial partnerships, such as entities often referred to as structured finance or special purpose entities, which would have been established for the purpose of facilitating off-balance sheet arrangements or for another contractually narrow or limited purpose.

Contractual Payment Obligations

In our continuing operations, we have entered into long-term contractual arrangements from time to time for our facilities, the provision of goods and services, and acquisition of technology access rights, among others. The following table presents contractual obligations arising from these arrangements as of December 31, 2016:

	Payment Due by Period				
	Total	Less Than 1 Year	1 - 3 Years	3 - 5 Years	More than 5 Years
	(in thousands)				
Senior Secured Term Loan (1)	$ 15,932	$ 10,818	$ 5,114	$ —	$ —
Long-term Mortgage Loans (1)	1,736	171	342	343	880
Lease obligations	20,174	4,737	9,829	5,441	167
Purchase obligations (2)	87,141	47,212	39,929	—	—
Total contractual obligations and contingencies	$ 124,983	$ 62,938	$ 55,214	$ 5,784	$ 1,047

(1) Interest is included.

(2) Purchase obligations include agreements to purchase goods or services that are enforceable and legally binding or subject to cancellation fees and that specify all significant terms. Purchase obligations relate primarily to our commercialization of EXONDYS 51 and clinical programs for DMD.

Milestone Obligations

For product candidates that are currently in various research and development stages, we may be obligated to make up to $712.6 million of future development, up-front royalty and sales milestone payments associated with our collaboration and license agreements. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory or sales milestones. Because the achievement of these milestones had not occurred as of December 31, 2016, such contingencies have not been recorded in our 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 sales milestones.

Other Funding Commitments

As of December 31, 2016, we have several on-going clinical studies in various clinical trial stages. Our most significant clinical trial expenditures are to contract research organizations ("CROs"). The CRO contracts are generally cancellable at our option. As of December 31, 2016, we have approximately $65.3 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, government and government agency bonds and high-grade corporate bonds with maturities of 36 months or less. Our cash is deposited in and invested through highly rated financial institutions in North America. As of December 31, 2016, we had $329.3 million of cash, cash equivalents and investments, comprised of $122.4 million of cash and cash equivalents, $195.4 million short-term investments and $11.5 million of restricted cash and investments. The fair value of cash equivalents and short-term 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. For each of the years ended December 31, 2016 and 2015, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of less than $0.1 million to our interest rate sensitive instruments.

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, 2016. In making this assessment, management used the criteria set forth by the *Committee of Sponsoring Organizations of the Treadway Commission* ("COSO") in its 2013 Internal Control Integrated Framework.

Based on this assessment, management has concluded that, as of December 31, 2016, 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, 2016 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

In September 2016, we began shipping and recording revenue and inventory related to EXONDYS 51. In addition, we are using a third party logistics provider for shipping, finished goods inventory management, customer service, and certain other logistical and financial reporting services related to these shipments of EXONDYS 51. As a result, we are relying on their systems and processes for the above functions. We have implemented a variety of internal control processes in various functional areas of our company to ensure that financial data related to EXONDYS 51 revenue and inventory activity has been correctly reflected in our financial statements. Additionally, we also evaluated the adequacy and effectiveness of the internal controls at our third party logistics provider. We are not aware of any material adverse impacts on our internal controls over financial reporting as a result of the implementation of these new controls.

There have not been other 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, 2016 that our certifying officers concluded materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders
Sarepta Therapeutics, Inc.:

We have audited Sarepta Therapeutics, Inc. and subsidiaries' internal control over financial reporting as of December 31, 2016, based on *Internal Control – Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). Sarepta Therapeutics, Inc. and subsidiaries' management is responsible 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 internal control over financial reporting based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit 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 audit also included performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

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.

In our opinion, Sarepta Therapeutics, Inc. and subsidiaries maintained, in all material respects, effective internal control over financial reporting as of December 31, 2016, based on criteria established in *Internal Control – Integrated Framework (2013)* issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO).

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the consolidated balance sheets of Sarepta Therapeutics, Inc. and subsidiaries as of December 31, 2016 and 2015, and the related consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2016, and our report dated February 28, 2017 expressed an unqualified opinion on those consolidated financial statements.

(signed) KPMG LLP

Cambridge, Massachusetts
February 28, 2017

Item 9B. Other Information.

None.

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 2017 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 2017 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 2017 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 2017 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 2017 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:

(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:

		Incorporated by Reference to Filings Indicated				
Exhibit Number	**Description**	**Form**	**File No.**	**Exhibit**	**Filing Date**	**Provided Herewith**
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	
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	Amended and Restated Bylaws.	8-K	001-14895	3.1	9/25/14	
4.1	Form of Specimen Certificate for Common Stock.	10-Q	001-14895	4.1	8/8/13	
4.2	Form of Common Stock Purchase Warrant, issued on January 30, 2009.	8-K	001-14895	4.4	1/30/09	
4.3	Form of Common Stock Purchase Warrant, issued on August 25, 2009.	8-K	001-14895	4.1	8/24/09	
10.1†	Employment Agreement with Patrick Iversen, Ph.D., dated July 14, 1997.	10KSB	000-22613	10.12	3/30/98	
10.2†	Amendment to Employment Agreement with Patrick Iversen, Ph.D., dated December 28, 2008.	10-K	001-14895	10.5	3/15/11	
10.3†	Amendment No. 2 to Employment Agreement with Patrick Iversen, Ph.D., dated January 18, 2010.	10-K	001-14895	10.6	3/15/11	
10.4†	Amended and Restated Executive Employment Agreement dated April 19, 2013 by and between Sarepta Therapeutics, Inc. and Christopher Garabedian.	10-Q	001-14895	10.2	5/9/13	

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.5†	Executive Employment Agreement dated January 10, 2011 by and between AVI BioPharma, Inc. and Effie Toshav.	10-Q	001-14895	10.1	5/10/11	
10.6†	Executive Employment Agreement dated March 29, 2011 by and between AVI BioPharma, Inc. and Peter S. Linsley, Ph.D.	10-Q	001-14895	10.4	5/10/11	
10.7†	Executive Employment Agreement dated June 13, 2011 by and between AVI BioPharma, Inc. and Edward Kaye, M.D.	10-Q	001-14895	10.4	8/8/11	
10.8†	Stand Alone Stock Option Grant between AVI BioPharma, Inc. and Effie Toshav dated January 10, 2011.	10-Q	001-14895	10.2	5/10/11	
10.9†	Stand Alone Stock Option Grant between the Registrant and Peter Linsley dated May 16, 2011.	S-8	333-175031	4.8	6/20/11	
10.10†	Stand Alone Stock Option Grant between the Registrant and Edward Kaye dated June 20, 2011.	S-8	333-175031	4.9	6/20/11	
10.11†	AVI BioPharma, Inc. 2002 Equity Incentive Plan.	Schedule 14A	001-14895	Appendix A	4/11/02	
10.12†	Sarepta Therapeutics, Inc. Amended and Restated 2011 Equity Incentive Plan.	8-K	001-14895	10.1	7/1/16	
10.13**	Form of Stock Option Award Agreement under the Amended and Restated 2011 Equity Incentive Plan.					X
10.14**	Form of Restricted Stock Agreement under the Amended and Restated 2011 Equity Incentive Plan.					X
10.15†	AVI BioPharma, Inc. Non-Employee Director Compensation Policy.	8-K	001-14895	10.85	10/1/10	
10.16†	Form of Indemnification Agreement.	8-K	001-14895	10.86	10/8/10	
10.17**	Form of Restricted Stock Unit Award Agreement under 2011 Equity Incentive Plan.					X
10.18**	Form of Stock Appreciate Right Award Agreement under the 2011 Equity Incentive Plan.					X
10.19†	Form of Senior Vice President Change in Control and Severance Agreement.	10-K	001-14895	10.19	3/15/13	
10.20†	Form of Vice President Change in Control and Severance Agreement.	10-K	001-14895	10.20	3/15/13	
10.21†	Sarepta Therapeutics, Inc. Amended and Restated 2013 Employee Stock Purchase Plan.	8-K	001-14895	10.2	7/1/16	
10.22†	Executive Employment Agreement with Jayant Aphale, Ph.D.	10-Q	001-14895	10.1	8/8/13	
10.23†	Retention and Severance Benefits Letter Agreement dated May 9, 2013 by and between the Company and Michael A. Jacobsen.	10-Q	001-14895	10.3	5/9/13	
10.24†	Offer Letter dated October 23, 2013 by and between Sarepta Therapeutics, Inc. and Sandesh Mahatme.	10-K	001-14895	10.24	3/3/14	

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.25†	Offer Letter dated October 23, 2012 by and between Sarepta Therapeutics, Inc. and David Tyronne Howton.	10-K	001-14895	10.25	3/3/14	
10.26†	Executive Inducement Stock Option Agreement between Arthur Krieg and Sarepta Therapeutics, Inc.	10-K	001-14895	10.26	3/3/14	
10.27†	Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan, as amended.	S-8	001-14895	4.4	2/25/16	
10.28	Form of Stock Option Award Agreement under 2014 Employment Commencement Incentive Plan	10-K	001-14895	10.28	3/3/14	
10.29*	Collaboration and License Agreement between Isis Pharmaceuticals and Ercole Biotech, Inc. dated May 16, 2003.	10-K	001-14895	10.78	3/16/10	
10.30*	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	
10.31*	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	
10.32	Agreement between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency dated May 5, 2009.	10-Q	001-14895	10.72	8/10/09	
10.33	Amendment of Contract between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency (contract no. HDTRA1-07-C-0010), effective May 29, 2009.	10-Q	001-14895	10.74	8/10/09	
10.34	Amendment of Contract between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency (contract no. HDTRA 1-07-C0010), effective September 30, 2009.	10-Q	001-14895	10.77	11/9/09	
10.35*	Amendment of Contract between AVI BioPharma, Inc. and the U.S. Defense Threat Reduction Agency (contract no HDTRA 1-09-C-0046), effective March 25, 2010.	10-Q	001-14895	10.81	5/10/10	
10.36*	Contract Number HDTRA1-10-C-0079 between Defense Threat Reduction Agency and AVI BioPharma, Inc. dated June 4, 2010.	10-Q	001-14895	10.84	8/9/10	
10.37*	Modification No. PZ0001 to Contract Number HDTRA1-10-C-0079 between Defense Threat Reduction Agency and AVI BioPharma, Inc. effective March 3, 2011.	10-Q	001-14895	10.3	5/10/11	
10.38*	Modification No. P00005 to Contract Number HDTRA1-10-C-0079 between Defense Threat Reduction Agency and AVI BioPharma, Inc. effective April 13, 2011.	10-Q	001-14895	10.1	8/8/11	
10.39*	Contract Number W9113M-10-C-0056 between U.S. Army Space and Missile Defense Command and AVI BioPharma, Inc. dated July 14, 2010.	10-Q	001-14895	10.86	11/9/10	

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.40*	Contract Number W911QY-12-C-0117 between U.S. Department of Defense's Joint Project Manager Transformational Medical Technologies and Sarepta Therapeutics, Inc. dated August 23, 2012.	10-Q	001-14895	10.1	11/7/12	
10.41*	Modification No. P00005 to Contract Number W9113M-10-C-0056 between U.S. Army Space and Missile Defense Command and AVI BioPharma, Inc. effective August 15, 2011.	10-Q/A	001-14895	10.3	2/15/12	
10.42*	Sponsored Research Agreement between AVI BioPharma, Inc. and Charley's Fund, Inc., effective October 12, 2007.	10-K	001-14895	10.58	3/17/08	
10.43*	First Amendment to Sponsored Research Agreement between AVI BioPharma, Inc. and Charley's Fund, Inc. dated June 2, 2009.	10-Q	001-14895	10.75	8/10/09	
10.44	Commercial Lease between Research Way Investments, Landlord, and Antivirals, Inc., Tenant, effective June 15, 1992.	SB-2	333-20513	10.9	1/28/97	
10.45	Lease Extension and Modification Agreement dated September 1, 1996, by and between Research Way Investments and Antivirals, Inc.	10-K	001-14895	10.53	3/15/11	
10.46	Second Lease Extension and Modification Agreement dated January 24, 2006 by and between Research Way Investments and AVI BioPharma, Inc.	10-Q	001-14895	10.55	8/9/06	
10.47	Real Property Purchase Agreement by and between WKL Investments Airport, LLC and AVI BioPharma, Inc., dated March 1, 2007, as amended.	10-Q	001-14895	10.61	8/9/07	
10.48	Lease Agreement between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc., dated November 23, 2011.	10-K	001-14895	10.42	3/13/12	
10.49	First Amendment to Lease Agreement dated December 22, 2011 between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc.	10-K	001-14895	10.43	3/13/12	
10.50	Second Amendment to Lease Agreement dated January 20, 2012 between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc.	10-K	001-14895	10.44	3/13/12	
10.51	Lease dated July 27, 2009 by and between BMR-3450 Monte Villa Parkway, LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.76	11/9/09	
10.52	First Amendment to Lease dated August 30, 2011 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.4	11/8/11	
10.53	Second Amendment to Lease dated January 31, 2012 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-K	001-14895	10.47	3/13/12	
10.54	Third Amendment to Lease dated May 31, 2012 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.2	8/7/12	
10.55	Lease dated October 20, 2010, by and between S/I North Creek VII LLC and AVI BioPharma, Inc.	10-K	001-14895	10.57	3/15/11	

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.56	Lease Agreement dated June 25, 2013 by and between Sarepta Therapeutics, Inc. and ARE-MA Region No. 38, LLC.	8-K	001-14895	10.1	7/1/13	
10.57	Purchase and Sale Agreement dated May 22, 2014 between Sarepta Therapeutics, Inc. and Eisai Inc.	10-Q	001-14895	10.1	8/7/14	
10.58	Offer Letter dated January 6, 2014 by and between Sarepta Therapeutics, Inc. and Arthur Krieg, M.D.	10-Q	001-14895	10.1	5/8/14	
10.59†	Employment Agreement dated September 20, 2016 between Sarepta Therapeutics, Inc. and Edward M. Kaye, M.D.	10-Q	001-14895	10.1	11/7/16	
10.60	Credit and Security Agreement between Sarepta Therapeutics, Inc. and MidCap Financial dated June 26, 2015	10-Q	001-14895	10.1	8/6/15	
10.61	Pledge Agreement between Sarepta Therapeutics, Inc. and MidCap Financial dated June 26, 2015	10-Q	001-14895	10.2	8/6/15	
10.62†	Separation and Consulting Agreement and General Release between Sarepta Therapeutics, Inc. and Christopher Garabedian entered into on June 30, 2015	10-Q	001-14895	10.3	8/6/15	
10.63†	Amendment No. 1 to the Sarepta Therapeutics, Inc. Amended and Restated 2011 Equity Incentive Plan	8-K	001-14895	10.1	6/30/15	
10.63†	License and Collaboration Agreement between Summit (Oxford) Ltd and Sarepta Therapeutics, Inc. dated October 3, 2016	10-Q	001-14895	10.2	11/7/16	
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 Interim Chief Executive Officer and Chief Medical Officer, Edward Kaye, MD, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company's Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1***	Certification of the Company's President, Chief Executive Officer and Chief Medical Officer, Edward Kaye, MD, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2***	Certification of the Company's Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	XBRL Instance Document.					X
101.SCH	XBRL Taxonomy Extension Schema Document.					X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.					X

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.					X

† Indicates management contract or compensatory plan, contract or arrangement.
* Confidential treatment has been granted for portions of this exhibit.
** Field herewith
*** Furnished herewith.

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, 2017

SAREPTA THERAPEUTICS, INC.

By: /s/ Edward Kaye, MD
Edward Kaye, MD
President, Chief Executive Officer and Chief Medical Officer

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Edward Kaye, MD and Sandesh Mahatme, 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, 2017:

Signature	Title
/s/ Edward Kaye, MD Edward Kaye, MD	President, Chief Executive Officer, Chief Medical Officer and Director (Principal Executive Officer)
/s/ Sandesh Mahatme Sandesh Mahatme	Senior 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/ Jean-Paul Kress, MD Jean-Paul Kress, MD	Director
/s/ Claude Nicaise, MD Claude Nicaise, MD	Director
/s/ Hans Wigzell Hans Wigzell, M.D., Ph.D.	Director

SAREPTA THERAPEUTICS, INC.
CONSOLIDATED FINANCIAL STATEMENTS

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders
Sarepta Therapeutics, Inc.:

We have audited the accompanying consolidated balance sheets of Sarepta Therapeutics, Inc. and subsidiaries as of December 31, 2016 and 2015, and the related consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows for each of the years in the three year period ended December 31, 2016. These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the consolidated financial statements referred to above present fairly, in all material respects, the financial position of Sarepta Therapeutics, Inc. and subsidiaries as of December 31, 2016 and 2015, and the results of its operations and its cash flows for each of the years in the three year period ended December 31, 2016, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), Sarepta Therapeutics, Inc. and subsidiaries' internal control over financial reporting as of December 31, 2016, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO), and our report dated February 28, 2017 expressed an unqualified opinion on the effectiveness of the Company's internal control over financial reporting.

(signed) KPMG LLP

Cambridge, Massachusetts
February 28, 2017

Sarepta Therapeutics, Inc.

Consolidated Balance Sheets

(in thousands, except share and per share amounts)

	As of December 31, 2016	As of December 31, 2015
Assets		
Current Assets:		
Cash and cash equivalents	$ 122,420	$ 80,304
Short-term investments	195,425	112,187
Accounts receivable	5,228	3,977
Inventory	12,813	—
Restricted investment	10,695	10,695
Other current assets	26,895	17,380
Total Current Assets	373,476	224,543
Restricted cash and investments	784	783
Property and equipment, net of accumulated depreciation of $30,346 and $24,594 as of December 31, 2016 and 2015, respectively	37,801	37,344
Intangible assets, net of accumulated amortization of $3,134 and $2,620 as of December 31, 2016 and 2015, respectively	8,076	6,642
Other assets	3,967	4,470
Total Assets	$ 424,104	$ 273,782
Liabilities and Stockholders' Equity		
Current Liabilities:		
Accounts payable	$ 29,690	$ 20,234
Accrued expenses	31,016	29,053
Current portion of long-term debt	10,108	5,936
Current portion of notes payable	—	2,493
Deferred revenue	3,303	3,303
Other current liabilities	1,305	1,275
Total Current Liabilities	75,422	62,294
Long-term debt	6,042	14,969
Deferred rent and other	5,949	6,172
Total Liabilities	87,413	83,435
Commitments and contingencies (Note 16)		
Stockholders' Equity:		
Preferred stock, $.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $.0001 par value, 99,000,000 shares authorized; 54,759,234 and 45,629,529 issued and outstanding at December 31, 2016 and 2015, respectively	5	5
Additional paid-in capital	1,503,126	1,089,508
Accumulated other comprehensive loss	(120)	(111)
Accumulated deficit	(1,166,320)	(899,055)
Total Stockholders' Equity	336,691	190,347
Total Liabilities and Stockholders' Equity	$ 424,104	$ 273,782

See accompanying notes to consolidated financial statements.

Sarepta Therapeutics, Inc.

Consolidated Statements of Operations and Comprehensive Loss

(in thousands, except per share data)

	For the Year Ended December 31,		
	2016	2015	2014
Revenues:			
Product, net	$ 5,421	$ —	$ —
Revenue from research contracts and other grants	—	1,253	9,757
Total revenues	5,421	1,253	9,757
Cost and expenses:			
Cost of sales	130	—	—
Research and development	188,272	146,394	94,231
Selling, general and administrative	83,749	75,043	49,315
Total cost and expenses	272,151	221,437	143,546
Operating loss	(266,730)	(220,184)	(133,789)
Other (loss) income:			
Interest (expense) income and other, net	(535)	154	779
Loss on change in warrant valuation	—	—	(2,779)
Total other (loss) income	(535)	154	(2,000)
Net loss	(267,265)	(220,030)	(135,789)
Other comprehensive loss:			
Unrealized loss on short-term securities - available-for-sale	(9)	(16)	(95)
Total other comprehensive loss	(9)	(16)	(95)
Comprehensive loss	$ (267,274)	$ (220,046)	$ (135,884)
Net loss per share — basic and diluted	$ (5.49)	$ (5.20)	$ (3.39)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	48,697	42,290	40,026

See accompanying notes to consolidated financial statements.

Sarepta Therapeutics, Inc.

Consolidated Statements of Stockholders' Equity

(in thousands)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
BALANCE AT DECEMBER 31, 2013	**37,752**	**$ 4**	**$ 790,424**	**$ —**	**$ (543,236)**	**$ 247,192**
Exercise of options for common stock	86	—	980	—	—	980
Exercise of warrants for common stock	766	—	19,536	—	—	19,536
Vest of restricted stock units	7	—	—	—	—	—
Shares withheld for taxes	(1)	—	(34)	—	—	(34)
Grant of restricted stock awards	6	—	—	—	—	—
Issuance of common stock for cash, net of offering costs	2,650	—	94,503	—	—	94,503
Issuance of common stock under employee stock purchase plan	46	—	1,015	—	—	1,015
Stock-based compensation	—	—	20,345	—	—	20,345
Unrealized loss from available-for-sale securities	—	—	—	(95)	—	(95)
Net loss	—	—	—	—	(135,789)	(135,789)
BALANCE AT DECEMBER 31, 2014	**41,312**	**4**	**926,769**	**(95)**	**(679,025)**	**247,653**
Exercise of options for common stock	817	—	10,010	—	—	10,010
Grant of restricted stock awards	181	—	—	—	—	—
Shares withheld for taxes	(6)	—	(182)	—	—	(182)
Issuance of common stock for cash, net of offering costs	3,250	1	119,915	—	—	119,916
Issuance of common stock under employee stock purchase plan	76	—	879	—	—	879
Stock-based compensation	—	—	32,117	—	—	32,117
Unrealized loss from available-for-sale securities	—	—	—	(16)	—	(16)
Net loss	—	—	—	—	(220,030)	(220,030)
BALANCE AT DECEMBER 31, 2015	**45,630**	**5**	**1,089,508**	**(111)**	**(899,055)**	**190,347**
Exercise of options for common stock	1,113	—	19,353	—	—	19,353
Grant of restricted stock awards, net of cancellations	50	—	—	—	—	—
Shares withheld for taxes	(47)	—	(2,168)	—	—	(2,168)
Issuance of common stock for cash, net of offering costs	7,876	—	364,749	—	—	364,749
Issuance of common stock under employee stock purchase plan	137	—	1,577	—	—	1,577
Stock-based compensation	—	—	30,107	—	—	30,107
Unrealized loss from available-for-sale securities	—	—	—	(9)	—	(9)
Net loss	—	—	—	—	(267,265)	(267,265)
BALANCE AT DECEMBER 31, 2016	**54,759**	**$ 5**	**$ 1,503,126**	**$ (120)**	**$ (1,166,320)**	**$ 336,691**

See accompanying notes to consolidated financial statements.

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

	For the Year Ended December 31,		
	2016	**2015**	**2014**
Cash flows from operating activities:			
Net loss	$ (267,265)	$ (220,030)	$ (135,789)
Adjustments to reconcile net income to cash flows in operating activities:			
Depreciation and amortization	5,611	5,247	3,690
Amortization of premium, net of discount, on available-for-sale securities and loss from sales of available-for-sale securities	80	652	2,432
Non-cash interest expense	355	367	12
Loss on abandonment of patents	293	197	128
Stock-based compensation	29,962	32,117	20,345
Non-cash restructuring expense	911	—	—
Increase in warrant valuation	—	—	2,779
Changes in operating assets and liabilities, net:			
(Increase) decrease in accounts receivable	(1,251)	(1,561)	1,114
Increase in inventory	(12,813)	—	—
(Increase) decrease in other assets	(9,012)	15,249	(34,013)
Increase in accounts payable, accrued expenses, deferred revenue and other liabilities	7,309	18,297	10,763
Net cash used in operations	(245,820)	(149,465)	(128,539)
Cash flows from investing activities:			
Release and maturity of restricted investments	—	—	7,250
Purchase of restricted investment	—	(10,695)	—
Purchase of property and equipment	(5,341)	(3,401)	(25,444)
Patent costs	(1,525)	(1,432)	(1,381)
Purchase of available-for-sale securities	(195,427)	(162,001)	(274,368)
Maturity and sales of available-for-sale securities	112,100	185,939	134,913
Net cash (used in) provided by investing activities	(90,193)	8,410	(159,030)
Cash flows from financing activities:			
Proceeds from borrowings, net of debt issuance costs	—	19,601	—
Repayments of long-term debt and notes payable	(7,603)	(2,598)	(94)
Proceeds from sales of common stock, net of offering costs	364,802	119,916	94,503
Proceeds from exercise of options and warrants and employee stock purchase program	20,930	10,889	9,746
Net cash provided by financing activities	378,129	147,808	104,155
Increase (decrease) in cash and cash equivalents	42,116	6,753	(183,414)
Cash and cash equivalents:			
Beginning of period	80,304	73,551	256,965
End of period	122,420	80,304	73,551
Supplemental disclosure of cash flow information:			
Cash paid during the period for interest	$ 1,562	$ 769	$ 77
Supplemental schedule of non-cash investing activities and financing activities:			
Accrued debt issuance costs related to the senior secured term loan	$ 400	$ 400	$ —
Property and equipment included in accrued expenses	$ 1,186	$ 318	$ 277
Intangible assets included in accrued expenses	$ 1,163	$ 335	$ 270
Issuance of common stock in satisfaction of warrants	$ —	$ —	$ 11,785
Shares withheld for taxes	$ 2,168	$ 182	$ 34
Issuance of notes payable in relation to the purchase of certain real and personal property located in Andover, Massachusetts	$ —	$ —	$ 4,613
Accrual for offering costs related to the equity offerings	$ 53	$ —	$ —

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 the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. Applying its proprietary, highly-differentiated and innovative platform technologies, the Company is able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy ("DMD") drug candidates. On September 19, 2016, the United States Food and Drug Administration ("FDA") granted accelerated approval for EXONDYS 51, indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 is studied in clinical trials under the name of eteplirsen and is marketed in the U.S. under the trademarked name of EXONDYS 51® (eteplirsen) Injection. In November 2016, the Company submitted a marketing authorization application ("MAA") for eteplirsen to the European Medicine Agency ("EMA") and the application was validated in December 2016.

As of December 31, 2016, the Company had approximately $329.3 million of cash, cash equivalents and investments, consisting of $122.4 million of cash and cash equivalents, $195.4 million of short-term investments and $11.5 million of restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments as of December 31, 2016 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 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 Therapeutics, Inc. and its wholly-owned subsidiaries. All intercompany transactions between and among its consolidated subsidiaries have been eliminated. Management has determined that the Company operates in one segment: discovering, developing, manufacturing and delivering therapies to patients with DMD. The Company's CEO, as the chief operating decision-maker, manages and allocates resources to the operations of the Company on a total company basis. 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 commercialization of EXONDYS 51 in the U.S. and internationally. 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.

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. Significant items subject to such estimates and assumptions include the valuation of stock-based awards, research and development expenses, income tax, inventory and revenue recognition.

Reclassification

The Company has revised the presentation as well as the caption of certain cash flows from financing activities within the consolidated statements of cash flows to conform to the current period presentation. "Proceeds from exercise of options and warrants and the sale of common stock, net of offering costs" of $130.8 million and $104.2 million for the years ended December 31, 2015 and 2014, respectively, have been reclassified into "Proceeds from sales of common stock, net of offering costs" and "Proceeds from exercise of options and warrants and employee stock purchase program" and presented separately on the consolidated statements of cash flows. This revision had no impact on net cash provided by financing activities or change in cash and cash equivalents.

The Company also has revised the presentation as well as the caption of certain accrued expenses in *Note 8, Accrued Expenses* to the consolidated financial statements to conform to the current period presentation. "Accrued facility-related costs" of $0.1 million as of December 31, 2015 is grouped into "Other". The reclassification had no impact on total current liabilities or total liabilities.

Additionally, the Company also has revised the presentation as well as the caption of net deferred tax assets in *Note 14, Income Taxes* to the consolidated financial statements to conform to the current period presentation. "Capitalized inventory" of $19.0 million as of December 31, 2015 is shown separately from "Net operating loss carryforwards" of $157.8 million and presented separately on the deferred tax assets table. The revision had no impact on net deferred tax assets or total assets.

Fair Value Measurements

The Company has certain financial assets 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 most of the Company's financial assets is categorized as Level 2 within the fair value hierarchy. These financial assets have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, through income-based approaches utilizing observable market data. For additional information related to fair value measurements, please read *Note 4, Fair Value Measurements* to the consolidated financial statements.

Cash and Cash Equivalents

Only investments that are highly liquid and readily convertible to cash and have original maturities of three months or less are considered cash equivalents. As of December 31, 2016, cash equivalents were comprised of money market funds.

Available-For-Sale Debt Securities

Available-for-sale debt securities are recorded at fair market value and unrealized gains and losses are included in accumulated other comprehensive loss in stockholder's equity. Realized gains and losses are reported in interest income and other, net, on a specific identification basis.

Accounts Receivable

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

The accounts receivable from product sales represents receivables due from the Company's specialty distributor and specialty pharmacies. 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 profile. The Company provides reserves against trade receivables for estimated 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, 2016, the credit profile for the Company's customers is deemed to be in good standing and write-offs of accounts receivable are not considered necessary. Historically, no accounts receivable amounts related to government research contracts and other grants have been written off and, thus, an allowance for doubtful accounts receivable related to government research contracts and other grants is not considered necessary.

The following table summarizes the components of the Company's accounts receivable for the period indicated:

	As of December 31, 2016	As of December 31, 2015
	(in thousands)	
Product sales	$ 4,002	$ —
Unbilled receivables	1,226	3,977
Total accounts receivable	$ 5,228	$ 3,977

The balance for unbilled receivables for both years presented is subject to government audit and will not be collected until the completion of the audit. The decrease in unbilled receivables is related to contract finalization and subsequent collection of the Ebola portion of the July 2010 Department of Defense contract.

Concentration of Credit Risk

Financial instruments which potentially subject the Company to concentrations of credit risk consist of accounts receivable from customers and cash, cash equivalent and short-term investments held at financial institutions.

For the year-ended December 31, 2016, the majority of the Company's accounts receivable arose from product sales in the U.S and all customers have standard payment terms which generally require payment within 30 to 45 days. Two individual customers accounted for 80% and 16% of net product revenues and 75% and 21% of accounts receivable from product sales, respectively. 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 profile. As of December 31, 2016, the Company believes that such customers are of high credit quality.

As of December 31, 2016, the Company's cash equivalents and short-term investments were concentrated at a single financial institution, which potentially exposes the Company to credit risks. The Company does not believe that there is significant risk of non-performance by the financial institution.

Inventory

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 which may be used in clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes.

The following table summarizes the components of the Company's inventory for the period indicated:

	As of December 31, 2016	As of December 31, 2015
	(in thousands)	
Raw materials	$ 9,531	$ —
Work in progress	3,175	—
Finished goods	107	—
Total inventory	$ 12,813	$ —

The Company periodically reviews 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 product is subject to strict quality control and monitoring which it performs throughout the manufacturing processes, certain batches or units of product may not meet quality specifications resulting in a charge to cost of sales.

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 its 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 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 equipment	5 years
Office equipment	5 years
Software and computer equipment	3 - 5 years
Leasehold improvements	Lesser of the useful life or the term of the respective lease
Land	Not depreciated
Building	30 years
Construction in Progress	Not depreciated until put into service

Intangible assets

The Company's intangible assets consist of an in-licensed right and patent costs, which are stated in the Company's consolidated balance sheets net of accumulated amortization and impairments, if applicable.

The in-licensed right relates to the license agreement with the University of Western Australia ("UWA"). As a result of the FDA approval and the subsequent commercial sale of EXONDYS 51, as defined in the Amended and Restated UWA License Agreement (*defined in Note 3*), the Company was obligated to pay a $1.0 million sales milestone to UWA and, accordingly, has recorded an in-licensed right. The in-licensed right will be 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 right. The amortization of the in-licensed right is recorded as cost of sales in the Company's consolidated statements of operations and comprehensive loss. As of December 31, 2016, the Company recorded less than $0.1 million cost of sales related to the amortization of the in-license right.

Patent costs consist primarily of external legal costs, filing fees incurred to file patent applications and renewal fees on proprietary technology developed or licensed by the Company. Patent costs associated with applying for a patent, being issued a patent and annual renewal fees are capitalized. Costs to defend a patent and costs to invalidate a competitor's patent or patent application are expensed as incurred. Patent costs are amortized on a straight-line basis over the shorter of the estimated economic lives or the initial term of the patents, which is generally 20 years. Patent amortization expense was $0.6 million, $0.5 million and $0.5 million for the years ended December 31, 2016, 2015 and 2014, respectively. The Company also expensed the remaining net book value of previously capitalized patents that were later abandoned of $0.3 million, $0.2 million and $0.1 million for the years ended December 31, 2016, 2015 and 2014, respectively, which were included in research and development expenses on the consolidated statements of operations and comprehensive loss.

The following table summarizes the estimated future amortization for intangible assets for the next five years:

	As of December 31, 2016 (in thousands)
2017	$ 705
2018	704
2019	698
2020	670
2021	652
Total	$ 3,429

Impairment of Long-Lived Assets

Long-lived assets held and used by the Company and intangible assets with definite lives are reviewed for impairment whenever events or circumstances indicate that the carrying amount of 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 such assets are 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.

Revenue Recognition

The Company recognizes revenue when all of the following criteria are met:

1) persuasive evidence of an arrangement exists;
2) delivery has occurred or services have been rendered;
3) price to the customer is fixed or determinable; and
4) collectability is reasonably assured.

Product revenues

Revenue from product sales is recognized when title and risk of loss have passed to the customer and is recorded net of applicable reserves for discounts and allowances.

Reserves for discounts and allowances

The Company establishes reserves for various government rebate and chargeback programs and co-payment assistance. Reserves established for these discounts and allowances are classified as either reductions of accounts receivable or a liability. These

reserves are based on estimates of the amounts earned or to be claimed on the related sales. Product revenue reserves represent the Company's estimates of outstanding claims for end-user rebate-eligible sales that have occurred, but for which related claim submissions have not been received. They are categorized as follows:

- Medicaid rebates relate to the Company's estimated obligations to states under established reimbursement arrangements. Rebate accruals 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 other current liabilities.
- Governmental chargebacks, including Public Health Service ("PHS") chargebacks, represent 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 established in the same period as the related revenue is recognized, resulting in a reduction in 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.
- Copay represents financial assistance to qualified patients, assisting them with prescription drug co-payments required by insurance. The copay 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 other current liabilities.

The Company also expects to maintain certain customer service contracts with distributors and other customers in the distribution channel that will provide inventory management, data and distribution services, which generally will be reflected as a reduction of revenue. To the extent the Company can demonstrate a separable benefit and fair value for these services, the Company will classify these payments as selling, general and administrative expenses.

Revenue from research contracts and other grants

The Company's contracts with the U.S. government are cost plus contracts providing for reimbursed costs which include overhead and general and administrative costs and a target fee. The Company recognizes revenue from government research contracts during the period in which the related expenses are incurred and presents such revenues and related expenses on a gross basis in the consolidated financial statements. The Company's government contracts are subject to government audits, which may result in catch-up adjustments. As of December 31, 2014, the Company had completed all development activities under its contracts with the U.S. government. The majority of the revenue under government contracts was recognized as of December 31, 2016 and only revenue for contract finalization, if any, is expected in the future.

Deferred revenue

If a technology, right, product or service is separate and independent of our performance under other elements of an arrangement, the Company defers recognition of non-refundable up-front fees if it has continuing performance obligations when the technology, right, product or service conveyed in conjunction with the non-refundable fee has no utility to the licensee. In addition, if the Company has continuing involvement through research and development services that are required because of its know-how or because the services can only be performed by the Company, such up-front fees are deferred and recognized over the period of continuing involvement. As of December 31, 2016, the Company had deferred revenue of $3.3 million, which represents up-front fees which it may recognize as revenue upon settlement of certain obligations.

Research and Development

Research and development expenses consist of costs associated with research activities as well as those with the Company's product development efforts, conducting preclinical studies, 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. Nonrefundable advance payments for goods or services that will be used or rendered for future research and development activities pursuant to an executory contractual arrangement will be deferred and capitalized, and recognized as an expense as the related goods are delivered or the related services are performed. If the Company does not expect the goods to be delivered or services to be rendered, the advance payment capitalized will be charged to expense.

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 clinical programs include salaries, stock-based compensation and an allocation of its facility 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.

Stock-Based Compensation

The Company's stock-based compensation programs include stock options, restricted stock awards ("RSAs"), restricted stock units ("RSUs"), stock appreciation rights ("SARs") and employee stock purchase program ("ESPP"). The Company accounts for stock-based compensation using the fair value method.

The fair values of stock options and SARs are estimated on the date of grant using the Black-Scholes-Merton option-pricing model. The fair values of RSAs 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 vesting period of the grants. For stock awards with performance-vesting conditions, the Company does not recognize compensation expense until it is probable that the performance-vesting condition will be achieved.

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 values of stock purchase rights are estimated using the Black-Scholes-Merton option-pricing model. The fair value of the look-back provision plus the 15% discount is recognized on a graded-vesting basis as stock-based compensation expense over the purchase period.

Income Taxes

The Company follows the asset and liability method of accounting for income taxes. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the consolidated financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. 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. To date, the Company has not had any earnings in its non-U.S. subsidiaries.

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered and settled. A valuation allowance is recorded to reduce the net deferred tax asset to zero because 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.

Rent Expense

The Company's operating leases for its Cambridge, Massachusetts and Corvallis, Oregon facilities provide for scheduled annual rent increases throughout each lease's term. The Company recognizes the effects of the scheduled rent increases on a straight-line basis over the full term of the leases.

For the years ended December 31, 2016, 2015 and 2014, the Company recognized rent expense and occupancy costs of $5.6 million, $5.2 million and $4.4 million, respectively.

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.

Subsequent Events

On February 21, 2017, the Company entered into an agreement to sell its Rare Pediatric Disease Priority Review Voucher ("PRV"). The Company received the PRV when EXONDYS 51 was approved by the FDA for the treatment of patients with DMD amenable to exon 51 skipping. As part of the agreement, the Company will receive an up-front payment of $125.0 million upon the

closing of the transaction, which is subject to customary closing conditions and is expected to occur following expiration of the applicable U.S. antitrust clearance requirements. Subsequent events have been evaluated up through the date that these consolidated financial statements were filed and no other material subsequent events were identified.

Recent Accounting Pronouncements

In November 2016, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2016-18, "*Statement of Cash Flows: Restricted Cash*". The amendments in this update requires amounts generally described as restricted cash and restricted cash equivalents be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. ASU No. 2016-18 will be effective for fiscal years beginning after December 15, 2017, with early adoption permitted. As of December 31, 2016, the Company has not elected to early adopt this guidance but expect the adoption of this guidance to have an impact on its statements of cash flows. The cash and cash equivalents balance at beginning of period and end of period would have increased by approximately $11.5 million had the guidance been adopted as of December 31, 2016.

In August 2016, the FASB issued ASU No. 2016-15, "*Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments*". The amendments in this update clarify how certain cash receipts and cash payments are presented and classified in the statement of cash flows. ASU No. 2016-15 will be effective for fiscal years beginning after December 15, 2017, with early adoption permitted. As of December 31, 2016, the Company has not elected to early adopt this guidance but does not expect the adoption of this guidance to have any impact on its consolidated financial statements.

In March 2016, the FASB issued ASU No. 2016-09, "*Improvements to Employee Share-Based Payment Accounting*". The amendments in this update simplify several aspects of the accounting for share-based payment transactions, including income tax consequences, classification of awards as either equity or liabilities and classification on the statement of cash flows. ASU No. 2016-09 will be effective for fiscal years beginning after December 15, 2016, with early adoption permitted. This standard will be effective for the Company on January 1, 2017. The Company has determined that the adoption of this standard will not have any impact on its consolidated financial statements.

In February 2016, the FASB issued ASU No. 2016-02, "*Leases (Topic 842)*", which supersedes Topic 840, "*Leases*". Under the new guidance, a lessee should recognize assets and liabilities that arise from its leases and disclose qualitative and quantitative information about its leasing arrangements. ASU No. 2016-02 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The adoption of this standard is expected to have an impact on the amount of the Company's assets and liabilities. As of December 31, 2016, the Company has not elected to early adopt this guidance and is currently evaluating the potential impact that this standard will have on the Company's consolidated financial statements.

In November 2015, the FASB issued ASU No. 2015-17, "*Balance Sheet Classification of Deferred Taxes*". The new standard requires that deferred tax assets and liabilities be classified as noncurrent in a classified statement of financial position. ASU No. 2015-17 will be effective for fiscal years beginning after December 15, 2016, with early adoption permitted. This standard will be effective for the Company on January 1, 2017. The Company has determined that the adoption of this standard will not have any impact on its consolidated financial statements.

In July 2015, the FASB issued ASU No. 2015-11, "*Inventory (Topic 330): Simplifying the Measurement of Inventory"*. The new standard applies only to inventory for which cost is determined by methods other than last-in, first-out and the retail inventory method, which includes inventory that is measured using first-in, first-out or average cost. Inventory within the scope of this standard is required to be measured at the lower of cost and net realizable value. Net realizable value is the estimated selling prices in the ordinary course of business, less reasonably predictable costs of completion, disposal, and transportation. The new standard will be effective for fiscal years beginning after December 15, 2016. As of December 31, 2016, the Company has elected to early adopt this guidance. The adoption of this standard did not have any impact on the Company's consolidated financial statements.

In August 2014, the FASB issued ASU No. 2014-15, "*Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*". This update requires an entity's management to evaluate whether there are conditions or events, considered in the aggregate, that raise substantial doubt about the entity's ability to continue as a going concern within one year after the date that the financial statements are issued or available to be issued and to provide related disclosures. ASU No. 2014-15 will be effective for the fiscal years ending after December 15, 2016, with early adoption permitted. The Company has adopted this guidance as of December 31, 2016. Based on the Company's financial condition as of the date these consolidated financial statements were issued or available for issuance, the adoption of this guidance did not have any impact on the current period consolidated financial statements.

In May 2014, the FASB issued ASU No. 2014-09, "*Revenue from Contracts with Customers (Topic 606)*". This ASU supersedes the revenue recognition requirements in Accounting Standards Codification Topic 605, "*Revenue Recognition*". Under the new guidance, a company is required to recognize revenue when it transfers goods or renders services to customers at an amount that it expects to be entitled to in exchange for these goods or services. The new standard allows for either a full retrospective with or without practical expedients or a retrospective with a cumulative catch upon adoption transition method. This guidance was originally

intended to be effective for the fiscal years beginning after December 15, 2016, with early adoption not permitted. In August 2015, the FASB issued ASU No. 2015-14, "*Deferral of the Effective Date*", which states that the mandatory effective date of this new revenue standard will be delayed by one year, with early adoption only permitted in fiscal year 2017. During the second quarter of 2016, the FASB issued three amendments to the new revenue standard to address some application questions: ASU No. 2016-10, "*Identifying Performance Obligations and Licensing*", ASU No. 2016-11, "*Rescission of SEC Guidance Because of Accounting Standards Updates 2014-09*", and ASU No. 2016-12, "*Narrow-Scope Improvements and Practical Expedients*". In December 2016, the FASB issued ASU No. 2016-20, "*Technical Corrections and Improvements to Topic 606, Revenue from Contracts with Customers",* which amends certain narrow aspects of the guidance issued in ASU 2014-09 including guidance related to the disclosure of remaining performance obligations and prior-period performance obligations, as well as other amendments to the guidance on loan guarantee fees, contract costs, refund liabilities, advertising costs and the clarification of certain examples. These three amendments will be effective upon adoption of Topic 606. As of December 31, 2016, the Company has not yet determined which adoption method it will utilize or the effect that the adoption of this guidance will have on its consolidated financial statements. As the Company just launched its first Commercial product in September 2016, it expects that the financial impact from this guidance through December 31, 2016 will be immaterial.

3. LICENSE AND COLLABORATION AGREEMENTS

Summit (Oxford) Ltd.

On October 3, 2016, the Company entered into an exclusive Collaboration and License Agreement (the "Collaboration Agreement") with Summit (Oxford) Ltd. ("Summit") which grants the Company the exclusive right to commercialize products in Summit's utrophin modulator pipeline in the EU, Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States (the "Licensed Territory").

Under the terms of the Collaboration Agreement, the Company made an up-front payment of $40.0 million to Summit, with additional payments of up to $192.0 million based on achievement of certain development and regulatory milestones for ezutromid, a Summit product candidate in its utrophin modulator pipeline. For each of Summit's future generation small molecule utrophin modulators, the Company may be required to make up to $290.0 million in development and regulatory milestone payments. Additionally, on a product-by-product basis, the Company may be required to make up to $330.0 million in sales milestone payments. The Collaboration Agreement also grants the Company an option to expand the Licensed Territory ("Option Territory"). If the Company exercises this option, it will be liable for a one-time $10.0 million option fee as well as up to $7.0 million in regulatory milestone payments. For each licensed product in the Option Territory, the Company may be liable for up to $82.5 million in sales milestone payments. Additionally, the Company may be required to make tiered royalty payments ranging from a low to high teens percentage of net sales on a product-by-product basis in the Licensed Territory.

Under the Collaboration Agreement, a joint steering committee will be established to plan, monitor and coordinate future development activities for ezutromid and future generation small molecule utrophin modulators. Summit will be solely responsible for all research and development costs for the licensed products until December 31, 2017. Thereafter, Summit will be responsible for 55.0% of the budgeted research and development costs related to the licensed products in the Licensed Territory, and the Company will be responsible for 45.0% of such costs. Any costs in excess of 110.0% of the budgeted amount are borne by the party that incurred such costs. Summit is also obligated to spend a specified minimum amount on the research and development of certain licensed products prior to the end of 2019.

For the year ended December 31, 2016, the Company recorded the $40.0 million up-front payment to Summit as research and development expense in the consolidated statement of operations and comprehensive loss as ezutromid is currently in a Phase 2 clinical trial.

University of Western Australia

In April 2013, the Company and UWA entered into an agreement under which an existing exclusive license agreement between the Company and UWA was amended and restated (the "Amended and Restated UWA License Agreement"). The Amended and Restated UWA License Agreement grants the Company specific rights to the treatment of DMD by inducing the skipping of certain exons. EXONDYS 51 falls under the scope of the license agreement. Under the Amended and Restated UWA License Agreement, the Company may be required to make payments of up to $6.0 million in aggregate to UWA based on the successful achievement of certain development and regulatory milestones relating to EXONDYS 51 and up to five additional product candidates. The Company may also be obligated to make payments to UWA of up to $20.0 million upon the achievement of certain sales milestones. Additionally, the Company may also be 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 Amended and Restated UWA License Agreement. However, the Company has the option to purchase future royalties up-front. Under this option, prior to the First Amendment (defined below), the Company could elect to make a one-time royalty payment of $30.0 million to UWA.

In June 2016, the Company and UWA entered into the first amendment to the Amended and Restated UWA License Agreement (the "First Amendment"). Under the First Amendment, the Company was obligated to make an up-front payment of $7.0 million to UWA upon execution of the amendment. Under the terms of the First Amendment, UWA has waived certain rights and amended the timing of certain payments under the Amended and Restated UWA License Agreement, including lowering the up-front payment that is due by the Company upon exercise of the option to purchase future royalties up-front. Upon exercise of the option to purchase future royalties up-front, the Company would still be obligated to make up to $20.0 million in payments to UWA upon achievement of certain sales milestones.

For the years ended December 31, 2016 and 2015, the Company recorded $7.6 million and $0.2 million, respectively, relating to the development milestone and up-front payments to UWA as research and development expense in the consolidated statement of operations and comprehensive loss as the Amended and Restated UWA License Agreement and its First Amendment were entered into before the FDA approval of EXONDYS 51. The Company did not incur any milestone expense for the year ended December 31, 2014. Additionally, corresponding to the FDA approval and the subsequent commercial sale of EXONDYS 51, as defined in the Amended and Restated UWA License Agreement, the Company recorded a $1.0 million milestone payment as an in-license right in its consolidated balance sheet as of December 31, 2016. The amortization of the in-licensed right is recorded as cost of sales in the Company's consolidated statements of operations and comprehensive loss. As of December 31, 2016, the Company did not make any royalty payments but may be obligated to make these payments in the future.

4. FAIR VALUE MEASUREMENTS

The tables below present information about the Company's financial assets 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, 2016			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 1,147	$ 1,147	$ —	$ —
Commercial paper	69,304	—	69,304	—
Government and government agency bonds	105,287	—	105,287	—
Corporate bonds	20,834	—	20,834	—
Certificates of deposit	11,343	11,343	—	—
Total assets	$ 207,915	$ 12,490	$ 195,425	$ —

	Fair Value Measurement as of December 31, 2015			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 32,850	$ 32,850	$ —	$ —
Commercial paper	48,899	—	48,899	—
Government and government agency bonds	50,918	—	50,918	—
Corporate bonds	17,370	—	17,370	—
Certificates of deposit	11,343	11,343	—	—
Total assets	$ 161,380	$ 44,193	$ 117,187	$ —

The Company's assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds and certificates of deposit. Money market funds are publicly traded mutual funds and are presented as cash equivalents on the consolidated balance sheets as of December 31, 2016

The Company's assets with fair value categorized as Level 2 within the fair value hierarchy consist of commercial paper, government and government agency bonds and corporate bonds. These assets have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, through income-based approaches utilizing market observable data.

The carrying amounts reported in the consolidated balance sheets for cash and cash equivalents, accounts receivable and accounts payable approximate fair value because of the immediate or short-term maturity of these financial instruments. The carrying amounts for long-term debt approximate fair value based on market activity for other debt instruments with similar characteristics and comparable risk.

5. CASH, CASH EQUIVALENTS AND SHORT-TERM INVESTMENTS

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 as of December 31, 2016 and 2015 was approximately four months. The following tables summarize the Company's cash, cash equivalents and short-term investments for each of the periods indicated:

	As of December 31, 2016			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 122,420	$ —	$ —	$ 122,420
Commercial paper	69,355	—	(51)	69,304
Government and government agency bonds	105,340	—	(53)	105,287
Corporate bonds	20,850	—	(16)	20,834
Total assets	$ 317,965	$ —	$ (120)	$ 317,845
As reported:				
Cash and cash equivalents	$ 122,420	$ —	$ —	$ 122,420
Short-term investments	195,545	—	(120)	195,425
Total assets	$ 317,965	$ —	$ (120)	$ 317,845

	As of December 31, 2015			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 75,304	$ —	$ —	$ 75,304
Commercial paper	48,936	—	(37)	48,899
Government and government agency bonds	50,966	—	(48)	50,918
Corporate bonds	17,396	—	(26)	17,370
Total assets	$ 192,602	$ —	$ (111)	$ 192,491
As reported:				
Cash and cash equivalents	$ 80,304			$ 80,304
Short-term investments	112,298		(111)	112,187
Total assets	$ 192,602	$ —	$ (111)	$ 192,491

6. OTHER CURRENT ASSETS AND OTHER NON-CURRENT ASSETS

The following table summarizes the Company's other current assets for each of the periods indicated:

	As of December 31, 2016	As of December 31, 2015
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 23,604	$ 13,070
Other prepaids	2,377	3,109
Other	914	1,201
Total other current assets	$ 26,895	$ 17,380

The following table summarizes the Company's other non-current assets for each of the periods indicated:

	As of December 31, 2016	As of December 31, 2015
	(in thousands)	
Prepaid clinical expenses	$ 3,725	$ 4,228
Other	242	242
Total other non-current assets	$ 3,967	$ 4,470

7. PROPERTY AND EQUIPMENT

Property and equipment are recorded at historical cost, net of accumulated depreciation. The following table summarizes components of property and equipment, net for each of the periods indicated:

	As of December 31,	
	2016	2015
	(in thousands)	
Land	$ 4,158	$ 4,158
Building and improvements	12,729	12,718
Software and computer equipment	6,775	6,149
Lab equipment	14,149	12,873
Office equipment	3,028	2,762
Leasehold improvements	22,872	21,723
Construction in progress	4,436	1,555
Property and equipment, gross	68,147	61,938
Less: accumulated depreciation	(30,346)	(24,594)
Property and equipment, net	$ 37,801	$ 37,344

For the years ended December 31, 2016, 2015 and 2014, depreciation expense totaled $5.0 million, $4.7 million and $3.2 million, respectively. Included in the $30.3 million accumulated depreciation as of December 31, 2016 was $0.8 million accelerated depreciation for certain assets whose expected useful lives were shortened due to the Corvallis plan (*defined in Note 9*).

8. ACCRUED EXPENSES

The following table summarizes the Company's accrued expenses for each of the periods indicated:

	As of December 31, 2016	As of December 31, 2015
	(in thousands)	
Accrued clinical and preclinical costs	$ 10,033	$ 9,587
Accrued employee compensation costs	8,748	8,189
Accrued contract manufacturing costs	4,673	4,830
Accrued professional fees	2,799	4,258
Accrued research costs	1,186	629
Other	3,577	1,560
Total accrued expenses	$ 31,016	$ 29,053

9. RESTRUCTURING

In March 2016, the Company announced a long-term plan ("Corvallis plan") to consolidate all of the Company's operations to Massachusetts and reduce its workforce by approximately 19% as part of a strategic plan to increase operational efficiency. As part of the consolidation, research activities and some employees will transition to the Company's facilities in Andover and Cambridge, Massachusetts. As of December 31, 2016, the relocations and terminations were substantially completed.

The restructuring costs of the Corvallis plan consist of costs associated with its workforce reduction and facility consolidation. The workforce reduction costs primarily relate to employee severance and benefits. Facility consolidation costs are primarily associated with non-cancellable lease obligations as well as accelerated depreciation for certain assets whose expected useful lives are shortened due to the consolidation. As of December 31, 2016, the second floor of the two floors at the Corvallis facility was

vacated and closed and made available for sub-leasing. The first floor of the facility continues to be used by the Company's employees. Using a discounted cash flow methodology and based on monthly rent payments as well as estimated sublease income, the Company recorded approximately $1.4 million in restructuring expense related to the vacated space of non-cancellable lease obligation. As of December 31, 2016, the Company continues to be obligated to make $6.4 million of minimum lease payments and certain other contractual maintenance costs for the whole facility. The Company estimates remaining restructuring expenses of approximately $0.2 million related to workforce reduction costs, which is accrued as earned over the service period for each employee.

In August 2016, the Company implemented a restructuring plan in Cambridge, Massachusetts ("Cambridge plan") and reduced its workforce by approximately 6%. The restructuring costs associated with the Cambridge plan consist of costs associated with workforce reduction totaling $0.6 million. The Cambridge plan was completed as of October 31, 2016.

For the year ended December 31, 2016, the Company recognized $4.6 million as restructuring expenses, respectively, $2.3 of which related to workforce reduction.

The following table summarizes the restructuring costs by function for the period indicated:

	For the Year Ended December 31, 2016 (in thousands)		
	Cash	Non-cash (1)	Total
Research and development	$ 1,631	$ 382	$ 2,013
Selling, general and administrative	2,020	529	2,549
Total restructuring expenses	$ 3,651	$ 911	$ 4,562

(1) The non-cash restructuring expenses include the following:
   a. $0.8 million accelerated depreciation for certain assets
   b. $0.1 million acceleration of stock option vesting

The following table summarizes the restructuring reserve for the period indicated:

	For the Year Ended December 31, 2016 (in thousands)
Restructuring expenses incurred during the period	3,651
Amounts paid during the period	(2,063)
Restructuring reserve ending balance	$ 1,588

10. INDEBTEDNESS

Senior Secured Term Loan

On June 26, 2015, the Company entered into a credit and security agreement (the "Credit Agreement") with MidCap Financial that provides a senior secured term loan of $20.0 million. Obligations under the Credit Agreement are secured by substantially all of the Company's assets, excluding, without limitation, the Company's intellectual property, certain equity interests relating to foreign subsidiaries and all assets owned by foreign subsidiaries, among others.

Borrowings under the Credit Agreement bear interest at a rate per annum equal to 7.75%, with only interest payments due through June 30, 2016. In addition to paying interest on the outstanding principal under the Credit Agreement, the Company will pay an origination fee equal to 0.50% of the amount of the term loan when advanced under the Credit Agreement, as well as a final payment fee equal to 2.00% of the amount borrowed under the Credit Agreement when the term loan is fully repaid. Commencing on July 1, 2016 and continuing for the remaining twenty-four months of the facility, the Company is required to make monthly principal payments of approximately $0.8 million.

The Company may voluntarily prepay outstanding loans under the Credit Agreement at any time, provided that the amount is not less than the total of all of the credit extensions and other related obligations under the Credit Agreement then outstanding. In the event of a voluntary prepayment, the Company is obligated to pay a prepayment fee equal to 2.95% of the outstanding principal of such advance if the prepayment is made within twelve months after the closing date, or 2.00% of the outstanding principal of such advance if the prepayment is made on or after the date that is twelve months after the closing date.

The Credit Agreement contains affirmative covenants that include government compliance, reporting requirements, maintaining property, making tax payments, maintaining insurance and cooperating during litigation. Additionally, the Company is required to maintain a minimum cash balance as collateral within its operating bank account with cash and cash equivalents of no less than the greater of the outstanding principal amount or $15.0 million. Negative covenants include restrictions on asset dispositions, acquisitions, indebtedness, liens, dividends and share purchases, amendments to material contracts and other restrictions.

The Credit Agreement includes customary events of default, including cross defaults, a change of control and a material adverse change. Additionally, the Company's failure to be compliant with the affirmative or negative covenants or make payments when they become due will result in an event of default.

The following table summarizes the components of the long-term debt recorded for the period indicated:

	As of December 31, 2016
	(in thousands)
Principal outstanding	15,000
Unamortized debt issuance costs	(223)
Net carrying value of senior secured term loan	14,777

Long-term Mortgage Loans

The Company has two loans outstanding which bear interest at 4.75%, mature in February 2027 and are collateralized by the facility the Company owns in Corvallis, Oregon. At December 31, 2016, these loans had unpaid principal balances of $0.9 million and $0.5 million, for a total indebtedness of $1.4 million.

As of December 31, 2016, the Company recorded approximately $10.1 million as current portion of long-term debt and approximately $6.0 million as long-term debt on the consolidated balance sheets related to the senior secured term loan and the long-term mortgage loans.

The following table summarizes the total payments under the Company’s debt arrangements:

	Senior Secured Term Loan (1)	Long-term Mortgage Loans (1)	Total
		(in thousands)	
2017	$ 10,818	$ 171	$ 10,989
2018	5,114	171	5,285
2019	—	171	171
2020	—	171	171
2021	—	172	172
Thereafter	—	880	880
Total Payments	$ 15,932	$ 1,736	$ 17,668

(1) Interest is included

11. EQUITY FINANCING

In September 2016, the Company sold approximately 5.8 million shares of common stock through an underwritten public offering at a price of $59.75 per share. The Company received aggregate net proceeds of approximately $327.4 million from the offering net of commission and offering expenses of approximately $17.6 million.

In June 2016, the Company sold approximately 2.1 million shares of common stock through an underwritten public offering at a price of $17.84 per share. The implied underwriting discount and commission was $1.60 per share. The Company received aggregate net proceeds of approximately $37.3 million from the offering net of offering expense of approximately $0.2 million.

In October 2015, the Company sold approximately 3.3 million shares of common stock at an offering price of $39.00 per share. The Company received aggregate net proceeds of approximately $119.9 million, after deducting the underwriting discounts and offering related transaction costs of approximately $6.8 million.

In April 2014, the Company sold approximately 2.7 million shares of common stock at an offering price of $38.00 per share. The Company received aggregate net proceeds of approximately $94.5 million, after deducting the underwriting discounts and offering related transaction costs of $6.2 million.

12. STOCK-BASED COMPENSATION

In June 2011, the Company's stockholders approved the 2011 Equity Incentive Plan ("2011 Plan"). The 2011 Plan, which authorized 13.0 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 2016 and 2015, shareholders authorized the issuance of an additional 1.3 million and 1.7 million shares, respectively, of common stock under the 2011 Plan. As of December 31, 2016, 3.0 million shares of common stock remain available for future grant under the 2011 Plan.

In June 2013, the Company's stockholders approved the 2013 ESPP with approximately 0.3 million shares of common stock available to be issued. In June 2016, the Company's stockholders approved an additional approximately 0.3 million shares of common stock available to be issued to be added to the 2013 ESPP. As of December 31, 2016, 0.3 million shares of common stock remain available for future grant under the 2013 ESPP.

In September 2014, the Company initiated the 2014 Employment Commencement Incentive Plan ("2014 Plan") with approximately 0.6 million shares of common stock available to be issued. In October 2015, the 2014 Plan was increased by 1.0 million shares of common stock available to be issued. As of December 31, 2016, 1.1 million shares of common stock remain available for future grant under the 2014 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 were measured on the date of grant using the Black-Scholes-Merton option-pricing model, with the following assumptions:

	For the Year Ended December 31,		
	2016	2015	2014
Risk-free interest rate (1)	1.1 – 1.8%	1.1 – 1.7%	1.4 – 1.7 %
Expected dividend yield (2)	—	—	—
Expected terms (3)	4.2 – 4.8 years	4.7 – 5.0 years	4.7 – 4.9 years
Expected volatility (4)	78.2 – 137.1 %	94.3 – 111.1 %	93.0 – 103.0 %

(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 terms are estimated using historical exercise behavior.
(4) The expected volatility is estimated using a blend of calculated volatility of the Company's common stock over a historical period and 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.

Additionally, the Company is required to estimate potential forfeiture of stock grants and adjust stock-based compensation cost recorded accordingly. The estimate of forfeitures is adjusted over the requisite service period to the extent that actual forfeitures differ, or are expected to differ, from such estimates. Changes in estimated forfeitures are recognized through a cumulative catch-up in the period of change and impact the amount of stock compensation expense to be recognized in future periods.

The following tables summarize the Company's stock option activity for each of the periods indicated:

	For the Year Ended December 31,					
	2016		2015		2014	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Grants outstanding at beginning of the period	6,515,976	$ 23.91	5,216,203	$ 24.45	4,190,367	$ 23.46
Granted	1,285,051	14.89	2,830,078	20.28	1,694,560	25.67
Exercised	(1,056,821)	18.31	(816,696)	12.26	(86,007)	11.40
Cancelled	(1,307,255)	24.61	(713,609)	26.78	(582,717)	22.79
Grants outstanding at end of the period	5,436,951	$ 22.70	6,515,976	$ 23.91	5,216,203	$ 24.45
Grants exercisable at end of the period	2,942,624	$ 24.42	2,617,167	$ 23.85	2,019,514	$ 18.69
Grants vested and expected to vest at end of the period	5,116,409	$ 22.83	5,908,213	$ 23.94	4,462,100	$ 23.27

The weighted-average grant date fair value per share of stock options granted during the years ended December 31, 2016, 2015 and 2014 was $12.46, $14.98 and $18.59, respectively.

	Aggregate Intrinsic Value (in thousands)	Weighted Average Remaining Contractual Life (Years)
Options outstanding at December 31, 2016	$ 38,660	7.41
Options exercisable at December 31, 2016	$ 17,483	6.45
Options vested and expected to vest at December 31, 2016	$ 35,955	7.33

The following table summarizes the Company's stock options vested and exercised for each of the periods indicated:

	For the Year Ended December 31,		
	2016	2015	2014
	(in thousands)		
Aggregate grant date fair value of stock options vested	$ 30,651	$ 27,858	$ 17,672
Aggregate intrinsic value of stock options exercised	$ 30,610	$ 18,138	$ 1,497

Stock Options with Service- and Performance-based Conditions

In February 2016, the Company granted to executives approximately 0.3 million stock options with service- and performance-based conditions ("February 2016 performance grants"). Vesting is achieved based upon the FDA approval of EXONDYS 51 and submission of MAA for eteplirsen to the EMA and continuing service over a four-year period. As a result of both milestones achieved in 2016, 50% of the February 2016 performance grants were triggered to be vested immediately and the remaining 50% are subject to the remaining service conditions of the awards.

In June 2013, the Company granted to executives approximately 0.4 million stock options with service- and performance-based conditions ("June 2013 performance grants"). Vesting is achieved based upon various regulatory approval for EXONDYS 51 and filings of investigational new drug ("IND") submissions for other drug candidates and continuing service over a four-year period. Through the submission of two IND applications during 2014 and the approval of EXONDYS 51 in the U.S. in 2016, cumulatively, 80% of the June 2013 performance grants vested or are eligible to vest subject to the remaining service conditions of the awards. The remaining 20% were forfeited as the performance criteria was not met.

For the years ended December 31, 2016, 2015 and 2014, the Company has recognized approximately $5.0 million, $0.5 million and $1.2 million in stock-based compensation expense related to the options with performance-based criteria, respectively.

As of December 31, 2016, the total stock-based compensation expense related to non-vested awards with only service-vesting conditions not yet recognized is approximately $28.5 million and those with service- and performance-based conditions approximates $1.9 million.

Restricted Stock Awards

The Company grants RSAs to members of its board of directors and certain employees. The following table summarizes the Company's RSA activity for each of the periods indicated:

	For the Year Ended December 31,					
	2016		2015		2014	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Grants outstanding at beginning of the period	161,320	$ 21.50	6,000	$ 34.92	6,000	$ 34.92
Granted	117,553 (1)	41.22	181,783	20.80	—	—
Vested	(59,703)	13.62	(24,463)	20.21	—	—
Forfeited	(66,000) (2)	33.51	(2,000)	13.90	—	—
Grants outstanding at end of the period	153,170	$ 34.53	161,320	$ 21.50	6,000	$ 34.92

(1) In September 2016, the Company granted executives RSAs with certain sales targets. If the sales targets are achieved within the required time frame, the number of RSAs may be increased from 71,925 to 89,906 shares. If and when deemed probable that such performance milestones may be achieved within the required time frame, the Company may recognize up to $4.4 million of stock-based compensation related to these grants.

(2) In September 2015, the Company granted executives 65,000 RSAs with performance conditions. As a result of the performance conditions not being met, these grants were cancelled during 2016.

Stock Appreciation Rights

The Company issues SARs to employees on the same terms as options granted to employees. The grant date fair value of the SARs is determined using the same valuation assumptions as for stock options described above. Stock-based compensation expense is recognized on a straight-line basis over the vesting period of the SARs.

In August 2012, 70,000 SARs were granted to the Company's former President and CEO and have an exercise price of $10.08 per share. In November 2012, 100,000 SARs were granted to the Company's Senior Vice President and CFO and have an exercise price of $23.85 per share. The SARs are classified as equity as the agreements require settlement in shares of stock.

The following table summarizes the Company's SAR activity for each of the periods indicated:

	For the Year Ended December 31,					
	2016		2015		2014	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Grants outstanding at beginning of the period	170,000	$ 18.18	170,000	$ 18.18	170,000	$ 18.18
Exercised	(67,812)	10.08	—	—	—	—
Forfeited	(2,188)	10.08	—	—	—	—
Grants outstanding at end of the period	100,000	23.85	170,000	18.18	170,000	18.18
Grants exercisable at end of the period	100,000	$ 23.85	141,249	$ 17.59	92,916	$ 17.80
Grants vested and expected to vest at end of the period	100,000	$ 23.85	167,813	$ 18.29	170,000	$ 18.18

	Aggregate Intrinsic Value (in thousands)	Weighted Average Remaining Contractual Life (Years)
SARs outstanding, exercisable and vested at December 31, 2016	$ 358,000	5.85

2013 Employee Stock Purchase Plan

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 24-month award period will end between February 28, 2018 and August 31, 2018. The following table summarizes the Company's ESPP activity and expense for each of the periods indicated:

	For the Year Ended December 31,	
	2016	2015
Number of shares purchased	137,113	75,539
Proceeds received (in millions)	$ 1.6	$ 0.9

Stock-based Compensation Expense

For the years ended December 31, 2016, 2015 and 2014, total stock-based compensation expense was $30.0 million, $32.1 million and $20.3 million, respectively. Included in the amount for the year ended December 31, 2015 is $8.6 million of stock-based compensation expense incurred in connection with the resignation of the Company's former CEO. The following table summarizes stock-based compensation expense by function included within the consolidated statements of operations and comprehensive loss:

	For the Year Ended December 31,		
	2016	2015	2014
	(in thousands)		
Research and development	$ 9,499	$ 10,403	$ 8,269
Selling, general and administrative	20,463	21,714	12,076
Total stock-based compensation	$ 29,962	$ 32,117	$ 20,345

The following table summarizes stock-based compensation expense by grant type included within the consolidated statements of operations and comprehensive loss:

	For the Year Ended December 31,		
	2016	2015	2014
	(in thousands)		
Stock options	$ 26,320	$ 29,014	$ 18,388
Employee stock purchase plan	2,380	2,165	1,165
Restricted stock awards	872	446	204
Other	390	492	588
Total stock-based compensation	$ 29,962	$ 32,117	$ 20,345

13. 401 (K) PLAN

The Company sponsors a 401 (k) Plan ("the Plan") which is a defined contribution plan. It 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. For employees who started before January 1, 2016, matching contributions fully vest after one year of service. For employees who started on and after January 1, 2016, matching contributions vest on a straight line basis over three years. The expense related to the Plan primarily consists of the Company's matching contributions.

Expense related to the Plan totaled $1.2 million, $0.9 million and $0.6 million for the years ended December 31, 2016, 2015 and 2014, respectively.

14. INCOME TAXES

As of December 31, 2016, the Company had federal and state net operating loss carryforwards of $498.5 million and $425.4 million, respectively, available to reduce future taxable income, which expire between 2016 and 2036. Utilization of these net operating losses could be limited under Section 382 of the Internal Revenue Code and similar state laws based on ownership changes and the value of the Company's stock. Additionally, the Company has $30.9 million and $9.7 million of federal and state research and development credits, respectively, available to offset future taxable income. These federal and state research and development credits begin to expire between 2018 and 2036 and between 2016 and 2031, respectively. Approximately $43.5 million of the Company's carryforwards were generated as a result of deductions related to exercises of stock options. The principal differences between net operating loss carryforwards for tax purposes and the accumulated deficit result from timing differences related to depreciation, amortization, treatment of research and development costs, limitations on the length of time that net operating losses may be carried forward, and differences in the recognition of stock-based compensation.

The Company had net deferred tax assets of $274.1 million and $219.8 million at December 31, 2016 and 2015, respectively, primarily from U.S. federal and state net operating loss carryforwards, U.S. federal and state research and development tax credit carryforwards, stock-based compensation expense, capitalized inventory and intangibles. A valuation allowance was recorded to reduce the net deferred tax assets to zero because it is more likely than not that the deferred tax assets will not be realized.

An analysis of the deferred tax assets is as follows:

	As of December 31,	
	2016	2015
	(in thousands)	
Net operating loss carryforwards	$ 174,569	$ 138,786
Difference in depreciation and amortization	2,589	2,694
Research and development tax credits	37,812	31,397
Stock-based compensation	16,166	20,774
Deferred rent	2,306	2,771
Deferred revenue	1,315	1,324
Capitalized inventory	36,282	19,018
Other	3,047	3,060
Gross deferred tax assets	274,086	219,824
Valuation allowance	(274,086)	(219,824)
Net deferred tax assets	$ —	$ —

The net change in the valuation allowance for deferred tax assets was an increase of $54.2 million and $54.1 million for the years ended December 31, 2016 and 2015, respectively, mainly due to the increase in the net operating loss carryforwards, capitalized inventory and research and development tax credits.

The reconciliation between the Company's effective tax rate and the income tax rate is as follows:

	For the Year Ended December 31,		
	2016	2015	2014
Federal income tax rate	34.0 %	34.0 %	34.0 %
Research and development tax credits	1.5	0.3	2.4
Valuation allowance	(17.3)	(19.1)	(21.6)
Permanent Differences	(3.4)	(1.7)	(2.4)
Foreign rate differential	(14.8)	(13.5)	(12.4)
Effective tax rate	— %	— %	— %

Permanent differences affecting the Company's effective tax rate include stock-based compensation and losses in a foreign jurisdiction. In December 2012, the Company licensed certain intellectual property of Sarepta Therapeutics, Inc. to its wholly owned subsidiary, Sarepta International C.V. The parties also entered into a contract research agreement under which Sarepta Therapeutics, Inc. performs research services for Sarepta International C.V. In January 2016, Sarepta Therapeutics, Inc. entered into a manufacturing and distribution agreement as well as service agreement with Sarepta International C.V. For the years ended December 31, 2016 and 2015, Sarepta International C.V. incurred costs of $116.2 million and $87.4 million, respectively.

The reconciliation of the beginning and ending amount of total unrecognized tax benefits for the years ended December 31, 2016, 2015 and 2014 are as follows:

	For the Year Ended December 31,		
	2016	2015	2014
		(in thousands)	
Balance at beginning of the period	$ 3,706	$ —	$ —
Increase related to current year tax positions	801	613	—
Increase related to prior year tax positions	137	3,093	—
Balance at end of the period	$ 4,644	$ 3,706	$ —

The balance of total unrecognized tax benefits at December 31, 2016, if recognized, would not affect the effective tax rate on income from continuing operations, due to a full valuation allowance against the Company's deferred tax assets. The Company does not expect that the amount of unrecognized tax benefits to change materially in the next twelve months. The Company, including its domestic subsidiaries, files consolidated U.S. federal and state income tax returns. The Company's policy is to recognize interest and/or penalties related to income tax matters in income tax expense. The Company had no accrual for interest or penalties on its balance sheet at December 31, 2016 or 2015 and has not recognized interest and/or penalties in the statement of operations for years ended December 31, 2016, 2015 or 2014.

15. NET LOSS PER SHARE

Basic net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding. Diluted net loss per share is computed by dividing net loss 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 each of the periods presented, 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.

	For the Year Ended December 31,		
	2016	2015	2014
	(in thousands, except per share amounts)		
Net loss	(267,265)	$ (220,030)	$ (135,789)
Weighted-average number of shares of common stock and common stock equivalents outstanding:			
Weighted-average number of shares of common stock outstanding for computing basic loss per share	48,697	42,290	40,026
Dilutive effect of outstanding stock awards and stock options after application of the treasury stock method*	—	—	—
Weighted-average number of shares of common stock and dilutive common stock equivalents outstanding for computing diluted loss per share	48,697	42,290	40,026
Net loss per share — basic and diluted	$ (5.49)	$ (5.20)	$ (3.39)

* For the year ended December 31, 2016 and 2015, stock options, RSAs and SARs to purchase approximately 5.7 million and 6.8 million shares of common stock, respectively, were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

For the year ended 2014, stock options, RSAs, RSUs, SARs and warrants to purchase approximately 5.4 million shares of common stock were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

16. COMMITMENTS AND CONTINGENCIES

Lease Obligations

In June 2013, the Company entered into a lease agreement ("Cambridge lease") for its headquarters located in Cambridge, Massachusetts. As of December 31, 2016, the Company had entered into six amendments to the Cambridge lease, increasing its total rental space for its headquarters to 88,459 square feet. The Cambridge lease and its amendments will expire in January 2021. The agreement calls for a security deposit in the form of a letter of credit totaling $0.6 million. The Company purchased a certificate of deposit ("CD") to meet the requirement and it is recorded as a long-term restricted investment in the consolidated balance sheets as of December 31, 2016 and 2015.

In June 2014, the Company entered into an agreement to sublease from an unrelated third party 10,939 square feet of office space. The sublease was terminated on December 31, 2016.

In January 2014, the Company entered into an agreement to sublease 15,077 square feet of office space to an unrelated third party. The sublease expired in July 2015. In August 2015, the Company entered into an agreement to sublease this space to another unrelated third party. The sublease will expire in September 2017.

In February 2015, the Company entered into an agreement to sublease 7,461 square feet of office space to an unrelated third party. The sublease expired February 2016. In December 2016, the Company entered into an agreement to sublease this space to another unrelated third party. The sublease will expire in December 2017.

The Company also leases laboratory and office space in Corvallis, Oregon which will expire in December 2020. As of December 31, 2016, the Company vacated and exited one floor of its two floors at its facility in Corvallis, Oregon.

The following table summarizes the aggregate non-cancelable future minimum payments under the Company's leases:

	As of December 31, 2016 (in thousands)
2017	$ 4,737
2018	4,854
2019	4,975
2020	5,097
2021	344
Thereafter	167
Total minimum lease payments	$ 20,174

Royalty Obligations

The Company is obligated to pay royalties on net sales of certain of its products. The royalty rates are in the low to high teens percentages for both inside and outside the United States. For example, under the Collaboration Agreement with Summit signed in October 2016, the Company may be required to make tiered royalty payments ranging from a low to high teens percentage of net sales on a product-by-product basis in the Licensed Territory. Under the Amended and Restated License Agreement with UWA signed in April 2013, the Company may be obligated to pay a low-single-digit percentage of royalty on the net sales of products exceeding certain amounts, which includes EXONDYS 51.

Milestone Obligations

The Company has collaboration and license agreements for which it could be obligated to pay up-front, development, regulatory and commercial milestones as a product candidate proceeds from the submission of an IND application through approval for commercial sale and beyond. As of December 31, 2016, for product candidates that are currently in various research and development stages, the Company may be obligated to make up to $712.6 million of future development, up-front royalty and sales milestone payments associated with its collaboration and license agreements. For the years ended December 31, 2016, 2015 and 2014, the Company recognized approximately $47.6 million, $0.2 million and $0 relating to certain up-front and development milestone payments as research and development expense, respectively, under these agreements. For the year ended December 31, 2016, the Company also recorded $1.0 million as an in-licensed right corresponding to the first sale of EXONDYS 51.

Other Funding Commitments

As of December 31, 2016, the Company has several on-going clinical studies 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. For the years ended December 31, 2016, 2015 and 2014, the Company recognized approximately $8.1 million, $8.5 million and $4.4 million, respectively, for expenditures incurred by CROs.

Litigation

In the normal course of business, the Company may from time to time be named as a party to various legal claims, actions and complaints, including matters involving securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. For example, purported class action complaints were filed against the Company and certain of its officers in the U.S. District Court for the District of Massachusetts on January 27, 2014 and January 29, 2014. The complaints were consolidated into a single action (Corban v. Sarepta, et. al., No. 14-cv-10201) by order of the court on June 23, 2014. Plaintiffs' consolidated amended complaint, filed on July 21, 2014, asserted violations of Section 10(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act") and Securities and Exchange Commission Rule 10b-5 against the Company, and Chris Garabedian, Sandy Mahatme, and Ed Kaye ("Individual Defendants," and collectively with the Company, the "Corban Defendants"), and violations of Section 20(a) of the Exchange Act against the Individual Defendants. Plaintiffs alleged that the Corban Defendants made material misrepresentations or omissions during the putative class period of July 24, 2013 through November 12, 2013, regarding a data set for a Phase 2b study of eteplirsen and the likelihood of the FDA accepting the Company's new drug application for eteplirsen for review based on that data set. Plaintiffs sought compensatory damages and fees. On August 18, 2014, the Corban Defendants filed a motion to dismiss, which the Court granted on March 31, 2015. Plaintiffs subsequently sought leave to file a second amended complaint, which the Corban Defendants opposed. On September 2, 2015, the Court denied Plaintiffs' motion for leave to amend as futile. Plaintiffs filed a notice of appeal on September 29, 2015, seeking review of the Court's March 31, 2015 order dismissing the case and the Court's September 2, 2015 order denying leave to amend. On January 27, 2016, Plaintiffs filed in the district court a motion for relief from judgment pursuant to Federal Rule of Civil Procedure 60(b)(2), arguing that the FDA Briefing Document published on or about January 15, 2016, was material and would have changed the Court's ruling. On February 26, 2016, the First Circuit stayed the appeal pending the district court's ruling on the 60(b)(2) motion. Defendants opposed the 60(b)(2) motion, and on April 21, 2016, the

Court denied Plaintiffs' motion for relief from judgment. On May 19, 2016, Plaintiffs filed a motion to alter or amend the April 21, 2016 order pursuant to Federal Rule of Civil Procedure 59(e). On May 20, 2016, the Court denied Plaintiffs' motion, and Plaintiffs filed a notice of appeal of the Court's April 21, 2016 denial of their 60(b)(2) motion and May 20, 2016 denial of their 59(e) motion. On June 13, 2016, the First Circuit granted Plaintiffs' motion to consolidate the two appeals. Oral argument is scheduled for March 7, 2017. An estimate of the possible loss or range of loss cannot be made at this time.

Another complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 styled William Kader, Individually and on Behalf of All Others Similarly Situated v. Sarepta Therapeutics Inc., Christopher Garabedian, and Sandesh Mahatme (Kader v. Sarepta et.al 1:14-cv-14318). On March 20, 2015, Plaintiffs filed an amended complaint asserting violations of Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5 against the Company, and Chris Garabedian and Sandy Mahatme ("Individual Defendants," and collectively with the Company, the "Kader Defendants"), and violations of Section 20(a) of the Exchange Act against the Individual Defendants. Plaintiffs alleged that the Kader Defendants made material misrepresentations or omissions during the putative class period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company's data for submission of an NDA for eteplirsen and the likelihood of the FDA accepting the NDA based on that data. Plaintiffs sought compensatory damages and fees. The Kader Defendants moved to dismiss the amended complaint on May 11, 2015. On April 5, 2016, following oral argument on March 29, 2016, the Court granted Defendants' motion to dismiss. On April 8, 2016, Lead Plaintiffs filed a motion for leave to file an amended complaint, which Defendants opposed. On January 6, 2017, the Court denied Plaintiffs' motion for leave to amend and dismissed the case. Plaintiffs filed a notice of appeal on February 3, 2017. A briefing schedule has not yet been set. An estimate of the possible loss or range of loss cannot be made at this time.

On February 5, 2015, a derivative suit was filed in the 215th Judicial District of Harris County, Texas against the Company's Board of Directors (*David Smith, derivatively on behalf of Sarepta Therapeutics, Inc., v. Christopher Garabedian et al., No. 2015-06645*). The claims allege that Sarepta's directors caused Sarepta to disseminate materially false and/or misleading statements in connection with disclosures concerning the Company's submission of the NDA for eteplirsen. Plaintiff seeks unspecified compensatory damages, actions to reform and improve corporate governance and internal procedures, disgorgement of profits, benefits and other compensation obtained by the directors, and attorneys' fees. The parties have agreed to stay the case pending resolution of the *Corban* and *Kader* cases. An estimate of the possible loss or range of loss cannot be made at this time.

On March 16, 2016, a derivative suit was filed in the U.S. District Court for the District of Massachusetts against the Company's Board of Directors (*Dawn Cherry, on behalf of nominal defendant Sarepta Therapeutics, Inc., v. Behrens et al., No. 16-cv-10531*). The claims allege that the defendants authorized the Company to make materially false and misleading statements about the Company's business prospects in connection with its development of eteplirsen from July 10, 2013 to the present. Plaintiffs seek unspecified damages, actions to reform and improve corporate governance and internal procedures, and attorneys' fees. The parties have agreed to stay the case pending resolution of the Corban and Kader cases. An estimate of the possible loss or range of loss cannot be made at this time.

Additionally, on September 23, 2014, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (*Terry McDonald, derivatively on behalf of Sarepta Therapeutics, Inc., et al. v. Goolsbee et al., No. 10157*). The claims allege, among other things, that (i) the Company's non-employee directors paid themselves excessive compensation fees for 2013, (ii) that the compensation for the Company's former CEO, Christopher Garabedian, was also excessive and such fees were the basis for Mr. Garabedian's not objecting to or stopping the excessive fees for the non-employee directors and (iii) that the disclosure in the 2013 proxy statement was deficient. The relief sought, among others, includes disgorgement and rescindment of allegedly excessive or unfair payments and equity grants to Mr. Garabedian and the directors, unspecified damages plus interest, a declaration that the Company's Amended and Restated 2011 Equity Plan at the 2013 annual meeting was ineffective and a revote for approved amendments, correction of misleading disclosures and plaintiff's attorney fees. The parties have agreed to a Memorandum of Understanding concerning the settlement terms and do not believe that disposition of the McDonald suit will have a material financial impact on the Company. The parties are now engaged in the confirmatory discovery process that, when complete, will allow plaintiffs' counsel to represent to the court that the terms of the settlement are fair. Defendants have provided documents to plaintiffs, who are now in the process of reviewing the materials. An estimate of the possible loss or range of loss cannot be made at this time.

Purchase Commitments

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 these arrangements:

	As of December 31, 2016 (in thousands)
2017	47,212
2018	28,521
2019	11,408
Total purchase commitments	$ 87,141

In connection with an amendment to a supply agreement, in September 2015, the Company issued an irrevocable standby letter of credit totaling $10.7 million to a contract manufacturing vendor. The obligation secured by the letter of credit will be fulfilled upon full payment of all deposits and purchase payments by February 2017. To meet the requirement of the letter of credit, the Company purchased $10.7 million in a certificate of deposit with a September 2017 maturity date. The Company has recorded this $10.7 million as a restricted investment on the consolidated balance sheet as of December 31, 2016.

17. FINANCIAL INFORMATION BY QUARTER (UNAUDITED)

	2016 for Quarter Ended			
	December 31	September 30	June 30	March 31
	(in thousands)			
Revenues:				
Product, net	$ 5,421	$ —	$ —	$ —
Total revenues	5,421	—	—	—
Operating expenses:				
Cost of sales	130	—	—	—
Research and development	70,749	34,349	44,348	38,826
Selling, general and administrative	22,937	22,184	17,752	20,876
Total operating expenses	93,816	56,533	62,100	59,702
Operating loss	(88,395)	(56,533)	(62,100)	(59,702)
Other (loss) income:				
Interest expense and other, net	(57)	(209)	(201)	(68)
Total other loss	(57)	(209)	(201)	(68)
Net loss	$ (88,452)	$ (56,742)	$ (62,301)	$ (59,770)
Net loss per share—basic and diluted	$ (1.62)	$ (1.18)	$ (1.35)	$ (1.31)
Shares used in per share calculations—basic and diluted	54,619	48,254	46,157	45,697

	2015 for Quarter Ended			
	December 31	September 30	June 30	March 31
	(in thousands)			
Revenue from research contracts and other grants	$ 1,253	$ —	$ —	$ —
Operating expenses:				
Research and development	41,376	36,673	29,180	39,165
General and administrative	24,329	15,090	12,927	22,697
Total operating expenses	65,705	51,763	42,107	61,862
Operating loss	(64,452)	(51,763)	(42,107)	(61,862)
Other income (loss):				
Interest (expense) income and other, net	(229)	(176)	256	303
Total other (loss) income	(229)	(176)	256	303
Net loss	$ (64,681)	$ (51,939)	$ (41,851)	$ (61,559)
Net loss per share—basic and diluted	$ (1.44)	$ (1.25)	$ (1.01)	$ (1.49)
Shares used in per share calculations—basic and diluted	44,882	41,565	41,357	41,324

EXHIBIT 10.13

SAREPTA THERAPEUTICS, INC.

AMENDED AND RESTATED 2011 EQUITY INCENTIVE PLAN

STOCK OPTION AWARD AGREEMENT

NOTICE OF STOCK OPTION GRANT

Participant: [Name of Participant]

Address:

The above-named Participant (the "Participant") has been granted an Option (the "Option") to purchase the number of shares of Common Stock of Sarepta Therapeutics, Inc. (the "Company") set forth below (the "Shares"), pursuant and subject to the terms and conditions of the Amended and Restated 2011 Equity Incentive Plan (the "Plan") and this Stock Option Award Agreement, including this Notice of Stock Option Grant (the "Notice of Grant") and the Terms and Conditions of Stock Option Grant attached hereto as Exhibit A, (together, this "Award Agreement"), as follows:

Date of Grant	_____
Vesting Commencement Date	_____
Exercise Price per Share	$_____
Total Number of Shares subject to Option	_____
Total Exercise Price	$_____
Type of Option:	_____ Incentive Stock Option
	_____ Nonstatutory Stock Option
Term/Expiration Date:	_____

Vesting Schedule

Subject to the terms and conditions of the Plan and this Award Agreement, the Option will vest and become exercisable in accordance with the following vesting schedule, with the number of Shares that vest on the first vesting date being rounded up to the nearest whole share, the number of Shares that vest on any subsequent vesting date being rounded down to the nearest whole share and the Option becoming vested as to 100% of the Shares on the final vesting date:

The Option will vest as to 25% of the total number of Shares subject to the Option on the first anniversary of the Vesting Commencement Date (set forth above) and thereafter will vest as to 1/48 of the total number of Shares subject to the Option on each monthly anniversary of the Vesting Commencement Date, subject to Participant remaining a Service Provider from the Date of Grant (set forth above) through each such vesting date..

Notwithstanding the foregoing, in the event the Participant's relationship with the Company as a Service Provider terminates as a result of the Service Provider's death, Option will vest as to 100% of the Shares as of the date of such death.

Exercisability of Option Following Termination of Relationship as a Service Provider

In the event of a termination of the Participant's relationship with the Company as a Service Provider, to the extent vested immediately prior to such termination, the Option will remain exercisable until the earlier of (a) the expiration of the three-month period following such termination, in the case of a termination other than due to the Participant's death or Disability, or the expiration of the 12-month period following such termination, in the case of a termination due to the Participant's death or Disability, or (b) the Term/Expiration Date, and except to the extent previously exercised as permitted by

the Plan and this Award Agreement, will thereupon immediately terminate.

Agreements and Acknowledgements

The Participant has reviewed the Plan and this Award Agreement in their entirety, has had an opportunity to obtain the advice of counsel prior to executing this Award Agreement and fully understands all provisions of the Plan and Award Agreement. The Participant hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Administrator upon any questions relating to the Plan and this Award Agreement. The Participant further agrees to notify the Company upon any change in the residence address indicated above.

Further, the Participant acknowledges and agrees that (i) this Award Agreement may be executed in two or more counterparts, each of which will be an original and all of which together will constitute one and the same instrument, (ii) this Award Agreement may be executed and exchanged using facsimile, portable document format (PDF) or electronic signature, which, in each case, will constitute an original signature for all purposes hereunder, and (iii) such signature by the Company will be binding against the Company and will create a legally binding agreement when this Award Agreement is countersigned by the Participant.

PARTICIPANT:	SAREPTA THERAPEUTICS, INC.
Signature	By
Print Name	Title

EXHIBIT A

TERMS AND CONDITIONS OF STOCK OPTION GRANT

1. Grant of Option. The Company hereby grants to the Participant the Option to purchase the Shares at the exercise price per share (the "Exercise Price"), as each are set forth in the Notice of Grant that forms a part of this Agreement, pursuant and subject to the terms and conditions of the Plan and this Award Agreement.

If designated in the Notice of Grant as an Incentive Stock Option ("ISO"), the Option is intended to qualify as an ISO under Section 422 of the Internal Revenue Code of 1986, as amended (the "Code") and is granted to the Participant in connection with the Participant's employment by the Company or a "subsidiary corporation" of the Company, as such term is defined in Code Section 424. However, even if the Option is intended to be an ISO, to the extent that it exceeds the $100,000 rule of Code Section 422(d) it will be treated as a Nonstatutory Stock Option ("NSO"). Further, if for any reason the Option (or portion thereof) does not qualify as an ISO, then, to the extent of such nonqualification, such Option (or portion thereof) will be regarded as an NSO granted under the Plan. In no event will the Administrator, the Company or any Parent or Subsidiary or any of their respective employees or directors have any liability to Participant (or any other person) due to the failure of the Option to qualify for any reason as an ISO. If designated in the Notice of Grant as an NSO, the Option will not qualify as an ISO and is granted in connection with the Participant's employment by or service to the Company and its qualifying subsidiaries. For purposes of the immediately preceding sentence, a "qualifying subsidiary" means a subsidiary of the Company as to which the Company has a "controlling interest" as described in Treas. Regs. §1.409A-1(b)(5)(iii)(E)(1).

2. Vesting Schedule. The term "vest" as used herein with respect to the Option or any portion thereof means to become exercisable, and the term "vested" as applied to any outstanding portion of the Option means that the Option is then exercisable, subject in each case to the terms of the Plan and this Award Agreement. Unless earlier terminated, forfeited, relinquished or expired and subject to the Participant's continuous relationship with the Company as a Service Provider from the Date of Grant through each applicable vesting date, the Option will vest in accordance with the vesting provisions set forth in the Notice of Grant.

3. Exercise of Option; Termination of Relationship as a Service Provider.

(a) Right to Exercise. No portion of the Option may be exercised until such portion vests as set forth in this Award Agreement and may be exercised only in accordance with the Plan and the terms of this Award Agreement. The latest date on which the Option or any portion thereof may be exercised is the Term/Expiration Date and if not exercised by such date the Option, or any remaining portion thereof, will thereupon immediately terminate.

(b) Termination of Relationship as a Service Provider.

(i) Except as otherwise provided in any employment or change of control or similar individual agreement between the Company and the Participant and as provided in Section 3(b)(ii) below, if the Participant's relationship with the Company as a Service Provider ceases, the Option, to the extent not already vested will be immediately forfeited and any vested portion of the Option that is then outstanding will remain exercisable for the period set forth in the Notice of Grant.

(ii) In the event the Participant's relationship with the Company as a Service Provider terminates as a result of the Service Provider's death, the Option will vest as to 100% of the Shares as of the date of such death and will remain exercisable for the period set forth in the Notice of Grant.

(c) Method of Exercise. This Option may be exercised by delivery to the Company of an exercise notice, in the form attached as Exhibit B (the "Exercise Notice") or in such other form (including electronic) acceptable to the Administrator, signed (including by electronic signature) by the Participant (or, in the event of the death of the Participant, the Beneficiary (as defined below)). Each election to exercise must be received by the Company at its principal office or by such other party as the Administrator may prescribe and must be accompanied by payment in full (including any applicable tax withholdings) for the number of Shares in respect of which the Option is being exercised (the "Exercised Shares").

4. Method of Payment. Payment of the aggregate Exercise Price may be by any of the following, or a combination thereof, at the election of Participant.

(a) cash;

(b) check;

(c) consideration received by the Company under a formal cashless exercise program implemented by the Company in connection with the Plan; or

(d) surrender of other Shares which have a Fair Market Value on the date of surrender equal to the aggregate Exercise Price of the Exercised Shares, provided that accepting such Shares, in the sole discretion of the Administrator, will not result in any adverse accounting consequences to the Company.

5. Death of Participant. In the event of the death of the Participant, the Option may be exercised by the beneficiary named in the written designation (in a form acceptable to the Administrator) most recently filed with the Administrator by the Participant and not subsequently revoked, or if there is no such designated beneficiary, by the executor or administrator of the Participant's estate (in each case, the "Beneficiary"). Any distribution or delivery to be made to the Participant under this Agreement will, if the Participant is then deceased, be made to the Participant's Beneficiary. The exercise of the Option or any portion thereof by the Beneficiary and any distribution or delivery under this Agreement to the Beneficiary will be subject to the Company receiving appropriate proof of the right of the Beneficiary to exercise the Option or receive such distribution or delivery, as the case may be, as determined by the Administrator.

6. Tax Obligations.

(a) Withholding Taxes. The exercise of the Option will give rise to "wages" subject to withholding. The Participant expressly acknowledges and agrees that the Participant's rights hereunder, including the right to be issued Shares upon exercise, are subject to the Participant promptly paying to the Company in cash or by check (or by such other means as may be acceptable to the Administrator) all taxes required to be withheld. No Shares will be transferred pursuant to the exercise of the Option unless and until the person exercising the Option has remitted to the Company an amount in cash or by check sufficient to satisfy any federal, state, or local withholding tax requirements, or has made other arrangements satisfactory to the Administrator with respect to such taxes. The Participant authorizes the Company and its subsidiaries to withhold such amount from any amounts otherwise owed to the Participant, but nothing in this sentence may be construed as relieving the Participant of any liability for satisfying his or her obligation under the preceding provisions of this Section.

(b) Notice of Disqualifying Disposition of ISO Shares. If the Option is an ISO, and if Participant sells or otherwise disposes of any of the Shares acquired pursuant to the ISO on or before the later of (i) the date that is two years after the Grant Date, or (ii) the date that is one year after the date of exercise, the Participant will immediately notify the Company in writing of such disposition. The Participant agrees that the Participant may be subject to income tax withholding by the Company on the compensation income recognized by Participant in connection with such disposition.

7. No Guarantee of Continued Service. NEITHER THE GRANT OF THE OPTION, NOR THE ISSUANCE OF SHARES UPON EXERCISE OF THE OPTION, WILL GIVE THE PARTICIPANT ANY RIGHT TO BE RETAINED IN THE EMPLOY OR SERVICE OF THE COMPANY OR ANY OF ITS SUBSIDIARIES, AFFECT THE RIGHT OF THE COMPANY OR ANY OF ITS SUBSIDIARIES TO DISCHARGE THE PARTICIPANT AT ANY TIME, OR AFFECT ANY RIGHT OF THE PARTICIPANT TO TERMINATE HIS OR HER RELATIONSHIP WITH THE COMPANY AS A SERVICE PROVIDER AT ANY TIME.

8. Non-transferability of Option. This Option may not be transferred except as expressly permitted under Section 5 of this Award Agreement or Section 14 of the Plan.

9. Additional Conditions to Issuance of Stock. The Company will not be obligated to deliver any Shares under this Agreement until: (i) the Company is satisfied that all legal matters in connection with the issuance and delivery of such Shares have been addressed and resolved; (ii) if the outstanding Common Stock is at the time of delivery listed on any stock exchange or national market system, the shares to be delivered have been listed or authorized to be listed on such exchange or system upon official notice of issuance; and (iii) all conditions contained in this Award Agreement have been satisfied or waived. The Company may require, as a condition to the exercise of the Option or the delivery of Shares under the Option, such representations or agreements as counsel for the Company may consider appropriate to avoid violation of the Securities Act of 1933, as amended, or any applicable state or non-U.S. securities law.

10. Provisions of the Plan. This Award Agreement is subject in its entirety to all terms and provisions of the Plan, which is incorporated herein by reference. In the event of a conflict between one or more provisions of this Award Agreement and one or more provisions of the Plan, the provisions of the Plan will govern. Capitalized terms used and not defined in this Award Agreement will have the meaning set forth in the Plan. A copy of the Plan as in effect on the Date of Grant has been furnished to the Participant. By accepting, or being deemed to have accepted, all or any part of the Option, the Participant agrees to be bound by the terms of the Plan and this Award Agreement.

11. Recoupment Policy; Stock Ownership Guidelines. The Option and any Shares issued pursuant to exercise of the Option (or any portion of the Option) will be subject to the Company's Recoupment Policy and its Stock Ownership Guidelines, where applicable.

12. Electronic Delivery. The Company may decide to deliver any documents related to the Option awarded hereunder or future equity awards that may be awarded under the Plan by electronic means or request Participant's consent to participate in the Plan by electronic means. Participant hereby consents to receive such documents by electronic delivery and agrees to participate in the Plan through any on-line or electronic system established and maintained by the Company or another third party designated by the Company.

13. Address for Notices. Any notice to be given to the Company under the terms of this Award Agreement will be addressed to the Company at Sarepta Therapeutics, Inc., 215 First Street, Suite 7, Cambridge, MA 02142, or at such other address as the Company may hereafter designate in writing.

14. Captions. Captions provided herein are for convenience only and are not to serve as a basis for interpretation or construction of this Award Agreement.

15. Agreement Severable. In the event that any provision in this Award Agreement will be held invalid or unenforceable, such provision will be severable from, and such invalidity or unenforceability will not be construed to have any effect on, the remaining provisions of this Award Agreement.

16. Modifications to the Agreement. This Award Agreement (including all exhibits) and the Plan constitute the entire understanding of the parties on the subjects covered. The Participant expressly warrants that he or she is not accepting this Award Agreement in reliance on any promises, representations, or inducements other than those contained herein. The Administrator may at any time or times amend this Award Agreement for any purpose which may at the time be permitted by law; *provided, however*, that except as otherwise expressly provided herein or in the Plan the Administrator may not, without the Participant's consent, alter the terms of this Award Agreement so as to affect materially and adversely the Participant's rights under this Award Agreement. Notwithstanding anything to the contrary in the Plan or this Award Agreement, the Company reserves the right to revise this Award Agreement as it deems necessary or advisable, in its sole discretion and without the consent of Participant, to comply with Section 409A of the Code or to otherwise avoid imposition of any additional tax or income recognition under Section 409A of the Code in connection with the Option.

17. Limitation on Liability. Notwithstanding anything to the contrary in the Plan or this Agreement, neither the Company, nor any of its subsidiaries, nor the Administrator, nor any person acting on behalf of the Company, any of its subsidiaries, or the Administrator, will be liable to the Participant or to any Beneficiary by reason of any acceleration of income, or any additional tax (including any interest and penalties), asserted by reason of the failure of this Option award to satisfy the requirements of Section 422 of the Code or Section 409A of the Code or by reason of Section 4999 of the Code, or otherwise asserted with respect to this Option award.

18. Governing Law. This Award Agreement will be governed by the laws of the State of Delaware, without giving effect to the conflict of law principles thereof. For purposes of litigating any dispute that arises under the Option or this Award Agreement, the parties hereby submit to and consent to the jurisdiction of the State of Delaware, and agree that such litigation will be conducted in the state courts of Delaware, or the federal courts for the United States for the District of Delaware, and no other courts, where the Option is granted and/or to be performed.

EXHIBIT B

SAREPTA THERAPEUTICS, INC.

AMENDED AND RESTATED 2011 EQUITY INCENTIVE PLAN

EXERCISE NOTICE

Sarepta Therapeutics, Inc.
215 First Street
Suite 7
Cambridge, MA 02142

1. Exercise of Option. Effective as of today, , , the undersigned ("Purchaser") hereby elects to exercise the option (the "Option") to purchase [#] shares (the "Shares") of the Common Stock of Sarepta Therapeutics, Inc. (the "Company") under and pursuant to the Amended and Restated 2011 Equity Incentive Plan (the "Plan") and the Stock Option Award Agreement dated_____, 20__, (the "Award Agreement"). The aggregate purchase price for the Shares is $, as required by this Award Agreement. Capitalized terms used herein without definition shall have the meanings given in the Plan and, if not defined in the Plan, the Agreement.

2. Delivery of Payment. Purchaser herewith delivers to the Company the full purchase price of the Shares and any required tax withholding to be paid in connection with the exercise of the Option.

3. Representations of Purchaser. Purchaser acknowledges that Purchaser has received, read and understood the Plan and this Award Agreement and agrees to abide by and be bound by their terms and conditions. The Purchaser further acknowledges that he or she has received and reviewed a copy of the prospectus required by Part I of Form S-8 relating to shares of Common Stock that may be issued under the Plan.

4. Rights as Shareholder. Until the issuance (as evidenced by the appropriate entry on the books of the Company or of a duly authorized transfer agent of the Company) of the Shares, no right to vote or receive dividends or any other rights as a shareholder will exist with respect to the Shares subject to the Option, notwithstanding the exercise of the Option. The Shares so acquired will be issued to Purchaser as soon as practicable after exercise of the Option. No adjustment will be made for a dividend or other right for which the record date is prior to the date of issuance, except as provided in Section 15 of the Plan.

5. Tax Consultation. Purchaser understands that Purchaser may suffer adverse tax consequences as a result of Purchaser's purchase or disposition of the Shares issued upon the exercise of the Option. Purchaser represents that Purchaser has consulted with any attorneys and tax consultants Purchaser deems advisable in connection with the exercise of the Option, and the purchase or disposition of the Shares and that Purchaser is not relying on the Company for any tax advice.

6. Entire Agreement; Governing Law. The Plan and Award Agreement are incorporated herein by reference. This Exercise Notice, the Plan and the Award Agreement constitute the entire agreement of the parties with respect to the subject matter hereof and supersede in their entirety all prior undertakings and agreements of the Company and Purchaser with respect to the subject matter hereof, and may not be modified adversely to the Purchaser's interest except by means of a writing signed by the Company and Purchaser. This Exercise Notice is governed by the internal substantive laws, but not the choice of law rules, of the State of Delaware.

[Signature Page to Follow]

Submitted by:

PURCHASER:

Signature

Print Name

Residence Address:

Accepted by:

SAREPTA THERAPEUTICS, INC.

By

Title

Date Received

EXHIBIT 10.14

SAREPTA THERAPEUTICS, INC.

AMENDED AND RESTATED 2011 EQUITY INCENTIVE PLAN

RESTRICTED STOCK AGREEMENT

NOTICE OF RESTRICTED STOCK GRANT

Participant: [Name of Participant]

Address:

The above-named Participant (the "Participant") has been granted the number of shares (the "Shares") of restricted Common Stock of Sarepta Therapeutics, Inc. (the "Company") set forth below (the "Restricted Stock), pursuant and subject to the terms and conditions of the Amended and Restated 2011 Equity Incentive Plan (the "Plan") and this Restricted Stock Agreement, including this Notice of Restricted Stock Grant (the "Notice of Grant") and the Terms and Conditions of Restricted Stock Grant attached hereto as Exhibit A, (this "Agreement"), as follows:

Date of Grant _____

Vesting Commencement Date _____

Number of Restricted Shares Granted _____

Vesting Schedule

Subject to the terms and conditions of the Plan and this Agreement, the Restricted Stock will vest, and the Company's right to reacquire the Restricted Stock will lapse, in accordance with the following vesting schedule, with the number of Shares that vest on the first vesting date being rounded up to the nearest whole share, the number of Shares that vest on any subsequent vesting date being rounded down to the nearest whole share and 100% of the Shares becoming vested on the final vesting date:

[INSERT VESTING SCHEDULE]

Notwithstanding the foregoing, in the event the Participant's relationship with the Company as a Service Provider terminates as a result of the Service Provider's death, 100% of the Shares of Restricted Stock will vest as of the date of such death.

Agreements and Acknowledgements

The Participant has reviewed the Plan and this Agreement in their entirety, has had an opportunity to obtain the advice of counsel prior to executing this Agreement and fully understands all provisions of the Plan and this Agreement. The Participant hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Administrator upon any questions relating to the Plan and this Agreement. The Participant further agrees to notify the Company upon any change in the residence address indicated above.

Further, the Participant acknowledges and agrees that (i) this Agreement may be executed in two or more counterparts, each of which will be an original and all of which together will constitute one and the same instrument, (ii) this Agreement may be executed and exchanged using facsimile, portable document format (PDF) or electronic signature, which, in each case, will constitute an original signature for all purposes hereunder, and (iii) such

signature by the Company will be binding against the Company and will create a legally binding agreement when this Agreement is countersigned by the Participant.

PARTICIPANT	SAREPTA THERAPEUTICS, INC.
_____	_____
Signature	
_____	_____
Print Name	

EXHIBIT A

TERMS AND CONDITIONS OF RESTRICTED STOCK GRANT

1. Grant of Restricted Stock. The Company hereby grants to the Participant the number of Shares of Restricted Stock, as each are set forth in the Notice of Grant that forms a part of this Agreement, pursuant and subject to the terms and conditions of the Plan and this Agreement.

2. Vesting Schedule. The term "vest" as used herein with respect to any Share of Restricted Stock means the lapsing of the restrictions described herein with respect to such Share. Unless earlier terminated, forfeited, relinquished or expired and subject to the Participant's continuous relationship with the Company as a Service Provider from the Date of Grant through each applicable vesting date, the Shares of Restricted Stock will vest in accordance with the vesting provisions set forth in the Notice of Grant.

3. Forfeiture upon Termination of Relationship with the Company as a Service Provider; Death of Participant.

(a) Except as otherwise provided in any employment or change of control or similar individual agreement between the Company and the Participant, upon the termination of the Participant's relationship with the Company as a Service Provider for any reason other than the death of the Participant, any then outstanding and unvested shares of Restricted Stock acquired by the Participant hereunder will be automatically and immediately forfeited. The Participant hereby (i) appoints the Company or, if applicable, the Company's designated escrow or transfer agent, as his or her attorney-in-fact to take such actions as may be necessary or appropriate to effectuate a transfer of the record ownership of any such shares that are unvested and forfeited hereunder, (ii) agrees to deliver to the Company, or to the Company's designated escrow or transfer agent, as applicable, as a precondition to the issuance of any certificate or certificates with respect to unvested shares of Restricted Stock hereunder, one or more stock powers, endorsed in blank, with respect to such shares, and (iii) agrees to sign such other powers and take such other actions as the Company, or the Company's designated escrow or transfer agent, as applicable, may reasonably request to accomplish the transfer or forfeiture of any unvested shares of Restricted Stock that is forfeited hereunder.

(b) In the event the Participant's relationship with the Company as a Service Provider terminates as a result of the Service Provider's death, 100% of the Shares of Restricted Stock will vest as of the date of such death.

4. Retention of Certificates. Any certificates representing unvested Shares of Restricted Stock will be held by the Company. If unvested shares of Restricted Stock are held in book entry form, the Participant agrees that the Company may give stop transfer instructions to the depository to ensure compliance with the provisions hereof.

5. Legend. All certificates representing unvested Shares of Restricted Stock will contain a legend substantially in the following form:

> THE TRANSFERABILITY OF THIS CERTIFICATE AND THE SHARES OF STOCK REPRESENTED HEREBY ARE SUBJECT TO THE TERMS AND CONDITIONS (INCLUDING FORFEITURE) OF THE AMENDED AND RESTATED SAREPTA THERAPEUTICS, INC. 2011 EQUITY INCENTIVE PLAN AND A RESTRICTED STOCK AGREEMENT ENTERED INTO BETWEEN THE REGISTERED OWNER AND SAREPTA THERAPEUTICS, INC. COPIES OF SUCH PLAN AND AGREEMENT ARE ON FILE IN THE OFFICES OF SAREPTA THERAPEUTICS, INC.

As soon as practicable following the vesting of any such Shares of Restricted Stock the Company shall cause a certificate or certificates covering such Shares, without the aforesaid legend, to be issued and delivered to the Participant. If any Shares of Restricted Stock are held in book-entry form, the Company may take such steps as it deems necessary or appropriate to record and manifest the restrictions applicable to such Shares.

6. Dividends, Voting Rights, etc. The Participant will be entitled to (i) receive any and all dividends or other distributions paid with respect to those Shares of Restricted Stock of which he or she is the record owner on the record date for such dividend or other distribution, and (ii) vote any Shares of Restricted Stock of which he or she is

the record owner on the record date for such vote; *provided, however*, that any property (other than cash) distributed with respect to a Share of Restricted Stock (the "associated share") acquired hereunder, including without limitation a distribution of shares of Common Stock by reason of a stock dividend, stock split or otherwise, or a distribution of other securities with respect to an associated share, will be subject to the restrictions of this Agreement in the same manner and for so long as the associated share remains subject to such restrictions, and will be promptly forfeited if and when the associated share is so forfeited; *and further provided*, that the Administrator may require that any cash distribution with respect to the shares other than a normal cash dividend be placed in escrow or otherwise made subject to such restrictions as the Administrator deems appropriate to carry out the intent of the Plan.

7. Death of Participant. Any distribution or delivery to be made to the Participant under this Agreement will, if the Participant is then deceased, be made to the beneficiary named in the written designation (in a form acceptable to the Administrator) most recently filed with the Administrator by the Participant and not subsequently revoked, or if there is no such designated beneficiary, by the executor or administrator of the Participant's estate (in each case, the "Beneficiary"). Any distribution or delivery under this Agreement to a Beneficiary will be subject to the Company receiving appropriate proof of the right of the Beneficiary to receive such distribution or delivery, as the case may be, as determined by the Administrator.

8. Withholding of Taxes. The award or vesting of the Shares of Restricted Stock acquired hereunder, and the payment of dividends with respect to such Shares, may give rise to "wages" subject to withholding. The Participant expressly acknowledges and agrees that the Participant's rights hereunder are subject to the Participant promptly paying to the Company in cash or by check (or by such other means as may be acceptable to the Company, including, if the Administrator so determines, by the delivery of previously acquired shares of Common Stock or Shares acquired hereunder or by the withholding of amounts from any payment hereunder) all taxes required to be withheld in connection with such award, vesting or payment. No certificates representing Shares will be transferred by the Company or its designated escrow or transfer agent nor restrictions otherwise removed from such Shares pursuant to the vesting of Shares of Restricted Stock unless and until the Participant has remitted to the Company an amount in cash or by check sufficient to satisfy any federal, state, or local withholding tax requirements, or has made other arrangements satisfactory to the Administrator with respect to such taxes. The Participant authorizes the Company and its subsidiaries to withhold such amount from any amounts otherwise owed to the Participant, but nothing in this sentence may be construed as relieving the Participant of any liability for satisfying his or her obligation under the preceding provisions of this Section.

9. No Guarantee of Continued Service. NEITHER THE GRANT OF THE RESTRICTED STOCK, NOR THE ISSUANCE OF SHARES UPON THE VESTING OF ANY PORTION OF THE RESTRICTED STOCK, WILL GIVE THE PARTICIPANT ANY RIGHT TO BE RETAINED IN THE EMPLOY OR SERVICE OF THE COMPANY OR ANY OF ITS SUBSIDIARIES, AFFECT THE RIGHT OF THE COMPANY OR ANY OF ITS SUBSIDIARIES TO DISCHARGE THE PARTICIPANT AT ANY TIME, OR AFFECT ANY RIGHT OF THE PARTICIPANT TO TERMINATE HIS OR HER EMPLOYMENT OR SERVICE AT ANY TIME.

10. Sale of Vested Shares; Non-transferability of Shares. The Participant understands that he or she will be free to sell any Share of Restricted Stock once it has vested, subject to (i) satisfaction of any applicable tax withholding requirements with respect to the vesting or transfer of such Share; (ii) the completion of any administrative steps (for example, but without limitation, the transfer of certificates) that the Company may reasonably impose; and (iii) applicable requirements of federal and state securities laws. Unvested Shares of Restricted Stock may not be transferred except as expressly permitted under Section 7 of this Agreement or Section 14 of the Plan.

11. Additional Conditions to Issuance and Vesting of Shares. The Company will not be obligated to deliver any Shares under the Plan or to remove any restriction from Shares previously delivered hereunder until: (i) the Company is satisfied that all legal matters in connection with the issuance and delivery of such Shares have been addressed and resolved; (ii) if the outstanding Common Stock is at the time of delivery listed on any stock exchange or national market system, the shares to be delivered have been listed or authorized to be listed on such exchange or system upon official notice of issuance; and (iii) all conditions contained in this Agreement have been satisfied or waived. The Company may require, as a condition to the delivery of Shares under this Agreement or the vesting of

such Shares, such representations or agreements as counsel for the Company may consider appropriate to avoid violation of the Securities Act of 1933, as amended, or any applicable state or non-U.S. securities law.

12. Provisions of the Plan. This Agreement is subject in its entirety to all terms and provisions of the Plan, which is incorporated herein by reference. In the event of a conflict between one or more provisions of this Agreement and one or more provisions of the Plan, the provisions of the Plan will govern. Capitalized terms used and not defined in this Agreement will have the meaning set forth in the Plan. A copy of the Plan as in effect on the Date of Grant has been furnished to the Participant. By accepting, or being deemed to have accepted, all or any part of the Restricted Stock, the Participant agrees to be bound by the terms of the Plan and this Agreement.

13. Recoupment Policy; Stock Ownership Guidelines. This award of Restricted Stock and any Shares issued pursuant to this Agreement will be subject to the Company's Recoupment Policy and its Stock Ownership Guidelines, where applicable.

14. Electronic Delivery. The Company may decide to deliver any documents related to the Shares of Restricted Stock awarded hereunder or future awards of restricted stock that may be awarded under the Plan by electronic means or request Participant's consent to participate in the Plan by electronic means. Participant hereby consents to receive such documents by electronic delivery and agrees to participate in the Plan through any on-line or electronic system established and maintained by the Company or another third party designated by the Company.

15. Form S-8 Prospectus. The Participant acknowledges that he or she has received and reviewed a copy of the prospectus required by Part I of Form S-8 relating to shares of Common Stock that may be issued under the Plan.

16. Address for Notices. Any notice to be given to the Company under the terms of this Agreement will be addressed to the Company at Sarepta Therapeutics, Inc., 215 First Street, Suite 7, Cambridge, MA 02142, or at such other address as the Company may hereafter designate in writing.

17. Captions. Captions provided herein are for convenience only and are not to serve as a basis for interpretation or construction of this Agreement.

18. Agreement Severable. In the event that any provision in this Agreement will be held invalid or unenforceable, such provision will be severable from, and such invalidity or unenforceability will not be construed to have any effect on, the remaining provisions of this Agreement.

19. Modifications to the Agreement. This Agreement constitutes the entire understanding of the parties on the subjects covered. The Participant expressly warrants that he or she is not accepting this Agreement in reliance on any promises, representations, or inducements other than those contained herein. The Administrator may at any time or times amend this Agreement for any purpose which may at the time be permitted by law; provided, however, that except as otherwise expressly provided herein or in the Plan the Administrator may not, without the Participant's consent, alter the terms of this Agreement so as to affect materially and adversely the Participant's rights under this Agreement. This Agreement and the award of Restricted Stock hereunder is intended to be exempt from Code Section 409A. Notwithstanding anything to the contrary in the Plan or this Agreement, the Company reserves the right to revise this Agreement as it deems necessary or advisable, in its sole discretion and without the consent of Participant, to comply with Code Section 409A, or to otherwise avoid imposition of any additional tax or income recognition under Code Section 409A in connection to this award of Restricted Stock.

20. Limitation on Liability. Notwithstanding anything to the contrary in the Plan or this Agreement, neither the Company, nor any of its subsidiaries, nor the Administrator, nor any person acting on behalf of the Company, any of its subsidiaries, or the Administrator, will be liable to the Participant or to any Beneficiary by reason of any acceleration of income, or any additional tax (including any interest and penalties), asserted by reason of the failure of this award of Restricted Stock to satisfy the requirements of Section 409A of the Code or by reason of Section 4999 of the Code, or otherwise asserted with respect to this award of Restricted Stock.

21. Governing Law. This Agreement will be governed by the laws of the State of Delaware, without giving effect to the conflict of law principles thereof. For purposes of litigating any dispute that arises under this award of Restricted Stock or this Agreement, the parties hereby submit to and consent to the jurisdiction of the State of Delaware, and agree that such litigation will be conducted in the state courts of Delaware, or the federal courts for the United States for the District of Delaware, and no other courts, where this award of Restricted Stock is made and/or to be performed.

EXHIBIT 10.17

SAREPTA THERAPEUTICS, INC.
2011 EQUITY INCENTIVE PLAN
RESTRICTED STOCK UNIT AWARD AGREEMENT

NOTICE OF RESTRICTED STOCK UNIT GRANT

Participant: [Name of Participant]

Address:

The above-named Participant (the "Participant") has been granted the number of restricted stock units (the "RSUs") set forth below giving the Participant the conditional right to receive, without payment therefor, one share of Common Stock of Sarepta Therapeutics, Inc. (the "Company") with respect to each RSU forming part of the award, pursuant and subject to the terms and conditions of the Amended and Restated 2011 Equity Incentive Plan (the "Plan") and this Restricted Stock Unit Award Agreement, including this Notice of Restricted Stock Unit Grant (the "Notice of Grant") and the Terms and Conditions of Restricted Stock Unit Grant attached hereto as Exhibit A, (this "Agreement"), as follows:

Date of Grant _____

Vesting Commencement Date _____

Number of RSUs _____

Vesting Schedule

Subject to the terms and conditions of the Plan and this Agreement, the RSUs will vest, in accordance with the following vesting schedule, with the number of RSUs that vest on the first vesting date being rounded up to the nearest whole share, the number of RSUs that vest on any subsequent vesting date being rounded down to the nearest whole share and 100% of the RSUs becoming vested on the final vesting date:

[INSERT VESTING SCHEDULE]

Notwithstanding the foregoing, in the event the Participant's relationship with the Company as a Service Provider terminates as a result of the Service Provider's death, 100% of the RSUs will vest as of the date of such death.

Agreements and Acknowledgements

The Participant has reviewed the Plan and this Agreement in their entirety, has had an opportunity to obtain the advice of counsel prior to executing this Agreement and fully understands all provisions of the Plan and this Agreement. The Participant hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Administrator upon any questions relating to the Plan and this Agreement. The Participant further agrees to notify the Company upon any change in the residence address indicated above.

Further, the Participant acknowledges and agrees that (i) this Agreement may be executed in two or more counterparts, each of which will be an original and all of which together will constitute one and the same instrument, (ii) this Agreement may be executed and exchanged using facsimile, portable document format (PDF) or electronic signature, which, in each case, will constitute an original signature for all purposes hereunder, and (iii) such signature by the Company will be binding against the Company and will create a legally binding agreement when this Agreement is countersigned by the Participant.

[Signature Page to Follow]

PARTICIPANT:

Signature

Print Name

SAREPTA THERAPEUTICS, INC.

By

Title

EXHIBIT A

TERMS AND CONDITIONS OF RESTRICTED STOCK UNIT GRANT

1. Grant of Restricted Stock Units. The Company hereby grants to the Participant the number of RSUs set forth in the Notice of Grant that forms a part of this Agreement giving the Participant the conditional right to receive, without payment therefor, one share of Common Stock with respect to each RSU forming part of the award pursuant and subject to the terms and conditions of the Plan and this Agreement.

2. Vesting Schedule. Unless earlier terminated, forfeited, relinquished or expired and subject to the Participant's continuous relationship with the Company as a Service Provider from the Date of Grant through each applicable vesting date, the RSUs will vest in accordance with the vesting provisions set forth in the Notice of Grant.

3. Company's Obligation to Pay. Subject to Section 4 below, the Company shall, as soon as practicable upon the vesting of any portion of the RSUs awarded hereunder (but in no event later than 30 days following the date on which such RSUs vest), effect delivery of the Shares with respect to such vested RSUs to the Participant (or, in the event of the Participant's the Beneficiary (as defined below)).

4. Forfeiture upon Termination of Relationship with Company as a Service Provider; Death of Participant.

(a) Except as otherwise provided in any employment or change of control or similar individual agreement between the Company and the Participant, upon the termination of the Participant's relationship with the Company as a Service Provider for any reason other than the death of the Participant, any then outstanding and unvested RSUs will be automatically and immediately forfeited.

(b) In the event the Participant's relationship with the Company as a Service Provider terminates as a result of the Service Provider's death, 100% of the RSUs will vest as of the date of such death.

5. Death of Participant. Any delivery to be made to the Participant under this Agreement will, if the Participant is then deceased, be made to the beneficiary named in the written designation (in a form acceptable to the Administrator) most recently filed with the Administrator by the Participant and not subsequently revoked, or if there is no such designated beneficiary, by the executor or administrator of the Participant's estate (in each case, the "Beneficiary"). Any delivery under this Agreement to a Beneficiary will be subject to the Company receiving appropriate proof of the right of the Beneficiary to receive such distribution or delivery, as the case may be, as determined by the Administrator.

6. Withholding of Taxes. The vesting of the RSUs awarded hereunder will give rise to "wages" subject to withholding. The Participant expressly acknowledges and agrees that the Participant's rights hereunder, including the right to be issued Shares upon vesting of the RSUs, are subject to the Participant promptly paying to the Company in cash or by check (or by such other means as may be acceptable to the Administrator) all taxes required to be withheld. No Shares will be transferred pursuant to the vesting of the RSUs unless and until the Participant has remitted to the Company an amount in cash or by check sufficient to satisfy any federal, state, or local withholding tax requirements, or has made other arrangements satisfactory to the Administrator with respect to such taxes. The Participant authorizes the Company and its subsidiaries to withhold such amount from any amounts otherwise owed to the Participant, but nothing in this sentence may be construed as relieving the Participant of any liability for satisfying his or her obligation under the preceding provisions of this Section.

7. Rights as Shareholder. Until the RSUs vest and the Shares underlying such vested RSUs are issued (as evidenced by the appropriate entry on the books of the Company or of a duly authorized transfer agent of the Company), no right to vote or receive dividends or any other rights as a stockholder will exist with respect to the Shares subject to the RSUs. No adjustment will be made for a dividend or other right for which the record date is prior to the date the Shares are issued, except as provided in Section 15(a) of the Plan.

8. No Guarantee of Continued Service. NEITHER THE GRANT OF THE RSUS, NOR THE ISSUANCE OF SHARES UPON THE VESTING OF ANY PORTION OF THE RSUS, WILL GIVE THE PARTICIPANT ANY RIGHT TO BE RETAINED IN THE EMPLOY OR SERVICE OF THE COMPANY OR ANY OF ITS

SUBSIDIARIES, AFFECT THE RIGHT OF THE COMPANY OR ANY OF ITS SUBSIDIARIES TO DISCHARGE THE PARTICIPANT AT ANY TIME, OR AFFECT ANY RIGHT OF THE PARTICIPANT TO TERMINATE HIS OR HER EMPLOYMENT OR SERVICE AT ANY TIME.

9. Grant is Not Transferable. This award of RSUs may not be transferred except as expressly permitted under Section 5 of this Agreement or Section 14 of the Plan.

10. Additional Conditions to Issuance of Shares. The Company will not be obligated to deliver any Shares under this Agreement until: (i) the Company is satisfied that all legal matters in connection with the issuance and delivery of such Shares have been addressed and resolved; (ii) if the outstanding Common Stock is at the time of delivery listed on any stock exchange or national market system, the shares to be delivered have been listed or authorized to be listed on such exchange or system upon official notice of issuance; and (iii) all conditions in this Agreement have been satisfied or waived. The Company may require, as a condition to the delivery of Shares under this Agreement or the vesting of such Shares, such representations or agreements as counsel for the Company may consider appropriate to avoid violation of the Securities Act of 1933, as amended, or any applicable state or non-U.S. securities law.

11. Provisions of the Plan. This Agreement is subject in its entirety to all terms and provisions of the Plan, which is incorporated herein by reference. In the event of a conflict between one or more provisions of this Agreement and one or more provisions of the Plan, the provisions of the Plan will govern. Capitalized terms used and not defined in this Agreement will have the meaning set forth in the Plan. A copy of the Plan as in effect on the Date of Grant has been furnished to the Participant. By accepting, or being deemed to have accepted, all or any part of the RSUs, the Participant agrees to be bound by the terms of the Plan and this Agreement.

12. Recoupment Policy; Stock Ownership Guidelines. This award of RSUs and any Shares issued pursuant to this Agreement will be subject to the Company's Recoupment Policy and its Stock Ownership Guidelines, where applicable.

13. Electronic Delivery. The Company may decide to deliver any documents related to the RSUs awarded hereunder or future awards of restricted stock units that may be awarded under the Plan by electronic means or request Participant's consent to participate in the Plan by electronic means. Participant hereby consents to receive such documents by electronic delivery and agrees to participate in the Plan through any on-line or electronic system established and maintained by the Company or another third party designated by the Company.

14. Address for Notices. Any notice to be given to the Company under the terms of this Agreement will be addressed to the Company at Sarepta Therapeutics, Inc., 215 First Street, Suite 7, Cambridge, MA 02142, or at such other address as the Company may hereafter designate in writing.

15. Captions. Captions provided herein are for convenience only and are not to serve as a basis for interpretation or construction of this Agreement.

16. Agreement Severable. In the event that any provision in this Agreement will be held invalid or unenforceable, such provision will be severable from, and such invalidity or unenforceability will not be construed to have any effect on, the remaining provisions of this Agreement.

17. Modifications to the Agreement. This Agreement constitutes the entire understanding of the parties on the subjects covered. The Participant expressly warrants that he or she is not accepting this Agreement in reliance on any promises, representations, or inducements other than those contained herein. The Administrator may at any time or times amend this Agreement for any purpose which may at the time be permitted by law; *provided, however,* that except as otherwise expressly provided herein or in the Plan the Administrator may not, without the Participant's consent, alter the terms of this Agreement so as to affect materially and adversely the Participant's rights under this Agreement. This Agreement and the award of RSUs hereunder is intended to be exempt from Section 409A of the Code. Notwithstanding anything to the contrary in the Plan or this Agreement, the Company reserves the right to revise this Agreement as it deems necessary or advisable, in its sole discretion and without the consent of

Participant, to comply with Section 409A of the Code, or to otherwise avoid imposition of any additional tax or income recognition under Section 409A of the Code in connection with this award of RSUs.

18. Limitation on Liability. Notwithstanding anything to the contrary in the Plan or this Agreement, neither the Company, nor any of its subsidiaries, nor the Administrator, nor any person acting on behalf of the Company, any of its subsidiaries, or the Administrator, will be liable to the Participant or to any Beneficiary by reason of any acceleration of income, or any additional tax (including any interest and penalties), asserted by reason of the failure of this award of RSUs to satisfy the requirements of Section 409A of the Code or by reason of Section 4999 of the Code, or otherwise asserted with respect to this award of RSUs.

19. Governing Law. This Agreement will be governed by the laws of the State of Delaware, without giving effect to the conflict of law principles thereof. For purposes of litigating any dispute that arises under this award of RSUs or this Agreement, the parties hereby submit to and consent to the jurisdiction of the State of Delaware, and agree that such litigation will be conducted in the state courts of Delaware, or the federal courts for the United States for the District of Delaware, and no other courts, where this award of RSUs is made and/or to be performed.

EXHIBIT 10.18

SAREPTA THERAPEUTICS, INC.

AMENDED AND RESTATED 2011 EQUITY INCENTIVE PLAN

STOCK APPRECIATION RIGHT AWARD AGREEMENT (STOCK SETTLED)

NOTICE OF STOCK APPRECIATION RIGHT GRANT

Participant Name: [Name of Participant]

Address:

The above-named Participant (the "Participant") has been granted a stock appreciation right (the "SAR") relating to the number of shares of Common Stock of Sarepta Therapeutics, Inc., (the "Company") set forth below (the "Shares"), to be settled in Common Stock upon exercise, pursuant to the terms and conditions of the Amended and Restated 2011 Equity Incentive Plan (the "Plan") and this Stock Appreciation Right Award Agreement (Stock Settled), including this Notice of Stock Appreciation Right Grant (the "Notice of Grant") and the Terms and Conditions of Stock Appreciation Right Award attached hereto as Exhibit A (together, this "Award Agreement") as follows:

Grant Number	_____
Date of Grant	_____
Vesting Commencement Date	_____
Exercise Price per Share:	_____
Number of Shares of Stock relating to SAR:	_____
Total Exercise Price:	_____
Term/Expiration Date:	_____

Vesting Schedule

Subject to the terms and conditions of the Plan and this Award Agreement, this SAR will vest and become exercisable, in accordance with the following vesting schedule, with the number of Shares that vest on the first vesting date being rounded up to the nearest whole share, the number of Shares that vest on any subsequent vesting date being rounded down to the nearest whole share and the SAR becoming vested as to 100% of the Shares on the final vesting date:

[INSERT VESTING SCHEDULE]

Notwithstanding the foregoing, in the event the Participant's relationship with the Company as a Service Provider terminates as a result of the Service Provider's death, the SAR will vest as to 100% of the Shares as of the date of such death.

Exercisability of SAR Following Termination of Relationship as a Service Provider

In the event of a termination of the Participant's relationship with the Company as a Service Provider, to the extent vested immediately prior to such termination, the SAR will remain exercisable until the earlier of (a) the expiration of the three-month period following such termination, in the case of a termination other than due to the Participant's death or Disability, or the expiration of the 12-month period following such termination, in the case of a termination due to the Participant's death or Disability, or (b) the Term/Expiration Date, and except to the extent previously exercised as permitted by the Plan and this Award Agreement, will thereupon immediately terminate.

Agreements and Acknowledgements

The Participant has reviewed the Plan and this Agreement in their entirety, has had an opportunity to obtain the advice of counsel prior to executing this Agreement and fully understands all provisions of the Plan and Agreement. The Participant hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Administrator upon any questions relating to the Plan and this Agreement. The Participant further agrees to notify the Company upon any change in the residence address indicated above.

Further, the Participant acknowledges and agrees that (i) this Agreement may be executed in two or more counterparts, each of which will be an original and all of which together will constitute one and the same instrument, (ii) this Agreement may be executed and exchanged using facsimile, portable document format (PDF) or electronic signature, which, in each case, will constitute an original signature for all purposes hereunder, and (iii) such signature by the Company will be binding against the Company and will create a legally binding agreement when this Agreement is countersigned by the Participant.

PARTICIPANT	**SAREPTA THERAPEUTICS, INC.**
Signature	By
Print Name	Title

EXHIBIT A

TERMS AND CONDITIONS OF STOCK APPRECIATION RIGHT AWARD

1. Grant of Stock Appreciation Right. The Company hereby grants to the Participant the SAR relating to the number of Shares and having the exercise price per share (the "Exercise Price"), as each are set forth in the Notice of Grant that forms a part of this Agreement, to be settled in shares of Common Stock upon exercise pursuant and subject to the terms and conditions of the Plan and this Award Agreement. The SAR is granted in connection with the Participant's employment by or service to the Company and its qualifying subsidiaries. For purposes of the immediately preceding sentence, a "qualifying subsidiary" means a subsidiary of the Company as to which the Company has a "controlling interest" as described in Treas. Regs. §1.409A-1(b)(5)(iii)(E)(1).

2. Vesting Schedule. The term "vest" as used herein with respect to the SAR or any portion thereof means to become exercisable, and the term "vested" as applied to any outstanding portion of the SAR means that the Option is then exercisable, subject in each case to the terms of the Plan and this Award Agreement. Unless earlier terminated, forfeited, relinquished or expired and subject to the Participant's continuous relationship with the Company as a Service Provider from the Date of Grant through each applicable vesting date, the SAR will vest in accordance with the vesting provisions set forth in the Notice of Grant.

3. Exercise of SAR; Termination of Relationship as a Service Provider.

(a) Right to Exercise. No portion of the SAR may be exercised until such portion vests as set forth in this Agreement and may be exercised only in accordance with the Plan and the terms of this Agreement. The latest date on which the SAR or any portion thereof may be exercised is the Term/Expiration Date and if not exercised by such date the SAR, or any remaining portion thereof, will thereupon immediately terminate.

(b) Termination of Relationship as a Service Provider.

(i) Except as otherwise provided in any employment or change of control or similar individual agreement between the Company and the Participant and as provided in Section 3(b)(ii) below, if the Participant's relationship with the Company as a Service Provider ceases, the SAR, to the extent not already vested will be immediately forfeited and any vested portion of the SAR that is then outstanding will remain exercisable for the period set forth in the Notice of Grant.

(ii) In the event the Participant's relationship with the Company as a Service Provider terminates as a result of the Service Provider's death, the SAR will vest as to 100% of the Shares as of the date of such death and will remain exercisable for the period set forth in the Notice of Grant.

(c) Method of Exercise. This SAR may be exercised by delivery to the Company of an exercise notice, in the form attached as Exhibit B (the "Exercise Notice") or in such other form (including electronic) acceptable to the Administrator, signed (including by electronic signature) by the Participant (or, in the event of the death of the Participant, the Beneficiary (as defined below)). Each election to exercise must be received by the Company at its principal office or by such other party as the Administrator may prescribe and must be accompanied by payment in full of any tax withholdings due in connection with such exercise.

4. Payment upon Exercise. Upon exercise of the SAR, or any portion thereof, the Company shall issue to the Participant a number of Shares (rounded down to the nearest whole share) having a Fair Market Value (determined as of the date on which the SAR is exercised) equal to the product of (a) the number of Shares with respect to which the SAR is exercised, and (b) the excess, if any, of (i) the Fair Market Value per Share upon the date of such exercise over (ii) the Exercise Price.

5. Death of Participant. In the event of the death of the Participant, the SAR may be exercised by the beneficiary named in the written designation (in a form acceptable to the Administrator) most recently filed with the Administrator by the Participant and not subsequently revoked, or if there is no such designated beneficiary, by the executor or administrator of the Participant's estate (in each case, the "Beneficiary"). Any distribution or delivery to

be made to the Participant under this Agreement will, if the Participant is then deceased, be made to the Participant's Beneficiary. The exercise of the SAR or any portion thereof by the Beneficiary and any distribution or delivery under this Agreement to the Beneficiary will be subject to the Company receiving appropriate proof of the right of the Beneficiary to exercise the SAR or receive such distribution or delivery, as the case may be, as determined by the Administrator.

6. Withholding Taxes. The exercise of the SAR will give rise to "wages" subject to withholding. The Participant expressly acknowledges and agrees that the Participant's rights hereunder, including the right to be issued Shares upon exercise, are subject to the Participant promptly paying to the Company in cash or by check (or by such other means as may be acceptable to the Administrator) all taxes required to be withheld. No Shares will be transferred pursuant to the exercise of the SAR unless and until the person exercising the SAR has remitted to the Company an amount in cash or by check sufficient to satisfy any federal, state, or local withholding tax requirements, or has made other arrangements satisfactory to the Administrator with respect to such taxes. The Participant authorizes the Company and its subsidiaries to withhold such amount from any amounts otherwise owed to the Participant, but nothing in this sentence may be construed as relieving the Participant of any liability for satisfying his or her obligation under the preceding provisions of this Section.

7. Rights as Shareholder. Until the Shares are issued (as evidenced by the appropriate entry on the books of the Company or of a duly authorized transfer agent of the Company), no right to vote or receive dividends or any other rights as a stockholder will exist with respect to the Shares subject to the SAR, notwithstanding the exercise of the SAR. No adjustment will be made for a dividend or other right for which the record date is prior to the date the Shares are issued, except as provided in Section 15(a) of the Plan.

8. No Guarantee of Continued Service. NEITHER THE GRANT OF THE SAR, NOR THE ISSUANCE OF SHARES UPON EXERCISE OF THE SAR, WILL GIVE THE PARTICIPANT ANY RIGHT TO BE RETAINED IN THE EMPLOY OR SERVICE OF THE COMPANY OR ANY OF ITS SUBSIDIARIES, AFFECT THE RIGHT OF THE COMPANY OR ANY OF ITS SUBSIDIARIES TO DISCHARGE THE PARTICIPANT AT ANY TIME, OR AFFECT ANY RIGHT OF THE PARTICIPANT TO TERMINATE HIS OR HER RELATIONSHIP WITH THE COMPANY AS A SERVICE PROVIDER AT ANY TIME.

9. Non-transferability of SAR. This SAR may not be transferred except as expressly permitted under Section 5 of this Agreement or Section 14 of the Plan.

10. Additional Conditions to Issuance of Stock. The Company will not be obligated to deliver any Shares under Plan or to remove any restriction from Shares previously delivered hereunder until: (i) the Company is satisfied that all legal matters in connection with the issuance and delivery of such Shares have been addressed and resolved; (ii) if the outstanding Common Stock is at the time of delivery listed on any stock exchange or national market system, the shares to be delivered have been listed or authorized to be listed on such exchange or system upon official notice of issuance; and (iii) all conditions contained in this Agreement have been satisfied or waived. The Company may require, as a condition to the exercise of the SAR or the delivery of Shares under the SAR, such representations or agreements as counsel for the Company may consider appropriate to avoid violation of the Securities Act of 1933, as amended, or any applicable state or non-U.S. securities law.

11. Provisions of the Plan. This Agreement is subject in its entirety to all terms and provisions of the Plan, which is incorporated herein by reference. In the event of a conflict between one or more provisions of this Agreement and one or more provisions of the Plan, the provisions of the Plan will govern. Capitalized terms used and not defined in this Agreement will have the meaning set forth in the Plan. A copy of the Plan as in effect on the Date of Grant has been furnished to the Participant. By accepting, or being deemed to have accepted, all or any part of the SAR, the Participant agrees to be bound by the terms of the Plan and this Award Agreement.

12. Recoupment Policy; Stock Ownership Guidelines. The SAR and any Shares issued pursuant to exercise of the SAR (or any portion of the SAR) will be subject to the Company's Recoupment Policy and its Stock Ownership Guidelines, where applicable.

13. Electronic Delivery. The Company may decide to deliver any documents related to the SAR awarded hereunder or future equity awards that may be awarded under the Plan by electronic means or request Participant's

consent to participate in the Plan by electronic means. Participant hereby consents to receive such documents by electronic delivery and agrees to participate in the Plan through any on-line or electronic system established and maintained by the Company or another third party designated by the Company.

14. Address for Notices. Any notice to be given to the Company under the terms of this Agreement will be addressed to the Company at Sarepta Therapeutics, Inc., 215 First Street, Suite 7, Cambridge, MA 02142, or at such other address as the Company may hereafter designate in writing.

15. Captions. Captions provided herein are for convenience only and are not to serve as a basis for interpretation or construction of this Agreement.

16. Agreement Severable. In the event that any provision in this Agreement will be held invalid or unenforceable, such provision will be severable from, and such invalidity or unenforceability will not be construed to have any effect on, the remaining provisions of this Agreement.

17. Modifications to the Agreement. This Agreement (including all exhibits) and the Plan constitute the entire understanding of the parties on the subjects covered. The Participant expressly warrants that he or she is not accepting this Agreement in reliance on any promises, representations, or inducements other than those contained herein. The Administrator may at any time or times amend this Agreement for any purpose which may at the time be permitted by law; *provided, however,* that except as otherwise expressly provided herein or in the Plan the Administrator may not, without the Participant's consent, alter the terms of this Agreement so as to affect materially and adversely the Participant's rights under this Agreement. Notwithstanding anything to the contrary in the Plan or this Agreement, the Company reserves the right to revise this Agreement as it deems necessary or advisable, in its sole discretion and without the consent of Participant, to comply with Section 409A of the Code or to otherwise avoid imposition of any additional tax or income recognition under Section 409A of the Code in connection to this SAR.

18. Limitation on Liability. Notwithstanding anything to the contrary in the Plan or this Agreement, neither the Company, nor any of its subsidiaries, nor the Administrator, nor any person acting on behalf of the Company, any of its subsidiaries, or the Administrator, will be liable to the Participant or to any Beneficiary by reason of any acceleration of income, or any additional tax (including any interest and penalties), asserted by reason of the failure of this SAR award to satisfy the requirements of Section 409A of the Code or by reason of Section 4999 of the Code, or otherwise asserted with respect to this SAR award.

19. Governing Law. This Agreement will be governed by the laws of the State of Delaware, without giving effect to the conflict of law principles thereof. For purposes of litigating any dispute that arises under the SAR or this Agreement, the parties hereby submit to and consent to the jurisdiction of the State of Delaware, and agree that such litigation will be conducted in the state courts of Delaware, or the federal courts for the United States for the District of Delaware, and no other courts, where the SAR is granted and/or to be performed.

EXHIBIT B

SAREPTA THERAPEUTICS, INC.

AMENDED AND RESTATED 2011 EQUITY INCENTIVE PLAN

STOCK APPRECIATION RIGHT (STOCK SETTLED) EXERCISE NOTICE

Sarepta Therapeutics, Inc.
215 First Street
Suite 7
Cambridge, MA 02142

1. Exercise of Stock Appreciation Right. Effective as of today, _____, 20__, the undersigned ("Participant,") hereby elects to exercise the stock appreciation right (the "SAR") relating to _____ shares (the "Shares") of the Common Stock of Sarepta Therapeutics, Inc. (the "Company") under and pursuant to the Amended and Restated. 2011 Equity Incentive Plan (the "Plan") and the Stock Appreciation Right Award Agreement dated _____, 20__, (the "Agreement"). The aggregate exercise price for the Shares will be $_____, pursuant to the Agreement. Capitalized terms used herein without definition shall have the meanings given in the Plan and, if not defined in the Plan, the Agreement.

2. Delivery of Payment. Participant herewith delivers to the Company any required tax withholding to be paid in connection with the exercise of the SAR.

3. Representations of Participant. Participant acknowledges that Participant has received, read and understood the Plan and the Agreement. Participant agrees to abide by and be bound by their terms and conditions. The Purchaser further acknowledges that he or she has received and reviewed a copy of the prospectus required by Part I of Form S-8 relating to shares of Common Stock that may be issued under the Plan.

4. Rights as Shareholder. Until the issuance (as evidenced by the appropriate entry on the books of the Company or of a duly authorized transfer agent of the Company) of the Shares, no right to vote or receive dividends or any other rights as a shareholder will exist with respect to the Shares issued upon exercise of the SAR, notwithstanding the exercise of the SAR. The Shares so acquired will be issued to Participant as soon as practicable after exercise of the SAR. No adjustment will be made for a dividend or other right for which the record date is prior to the date of issuance, except as provided in Section 15 of the Plan.

5. Tax Consultation. Participant understands that Participant may suffer adverse tax consequences as a result of Participant's exercise of the SAR or the disposition of any Shares issued upon exercise of the SAR. Participant represents that Participant has consulted with any attorneys and tax consultants Participant deems advisable in connection with the exercise of the SAR and the disposition of the Shares and that Participant is not relying on the Company for any tax advice.

6. Entire Agreement; Governing Law. The Plan and the Agreement are incorporated herein by reference. This Exercise Notice, the Plan and the Agreement constitute the entire agreement of the parties with respect to the subject matter hereof and supersede in their entirety all prior undertakings and agreements of the Company and Participant with respect to the subject matter hereof, and may not be modified adversely to Participant's interest except by means of a writing signed by the Company and Participant. This Exercise Notice is governed by the internal substantive laws, but not the choice of law rules, of the State of Delaware.

[Signature Page to Follow]

Submitted by:	Accepted by:
PARTICIPANT:	SAREPTA THERAPEUTICS, INC.
_____	_____
Signature	By
_____	_____
Print Name	Title
Residence Address:	
_____	
_____	
	_____
	Date Received

EXHIBIT 21.1

Sarepta Therapeutics, Inc.

Subsidiaries of the Registrant

Name	Jurisdiction of Incorporation
ST International Holdings, Inc.	Delaware, USA
STIH Two, Inc.	Delaware, USA
Sarepta Securities Corp.	Massachusetts, USA
Sarepta International CV	Netherlands
AVI BioPharma International Limited	United Kingdom

EXHIBIT 23.1

Consent of Independent Registered Public Accounting Firm

The Board of Directors
Sarepta Therapeutics, Inc.:

We consent to the incorporation by reference in the registration statements (Nos. 333-105412, 333-109015, 333-133211, 333-138299, 333-150021, 333-160922, 333-180258, 333-184807, 333-209709, 333-45888, 333-68502, 333-86039, 333-86778, and 333-93135) on Form S-3 and (Nos. 333-101826, 333-172823, 333-175031, 333-192287, 333-199037, 333-209710, 333-213022, 333-34047, 333-49994, 333-49996) on Form S-8 of Sarepta Therapeutics, Inc. and subsidiaries of our reports dated February 28, 2017, with respect to the consolidated balance sheets of Sarepta Therapeutics, Inc. and subsidiaries as of December 31, 2016 and 2015, and the related consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2016, and the effectiveness of internal control over financial reporting as of December 31, 2016, which reports appear in the December 31, 2016 annual report on Form 10-K of Sarepta Therapeutics, Inc. and subsidiaries.

(signed) KPMG LLP

Cambridge, Massachusetts
February 28, 2017

EXHIBIT 31.1

CERTIFICATION

I, Edward Kaye, MD, 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, 2017

/s/ Edward Kaye, MD
Edward Kaye, MD
President, Chief Executive Officer, Chief Medical Officer
(Principal Executive Officer)

EXHIBIT 31.2

CERTIFICATION

I, Sandesh Mahatme, 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, 2017

/s/ Sandesh Mahatme
Sandesh Mahatme
Senior 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, Edward Kaye, MD, 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, 2016, 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, 2017

/s/ Edward Kaye, MD

Edward Kaye, MD
President, Chief Executive Officer
and Chief Medical 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, Sandesh Mahatme, 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, 2016, 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, 2017

/s/ Sandesh Mahatme

Sandesh Mahatme,
Senior 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.