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

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2023

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 No.)**
215 First Street, Suite 415 Cambridge, MA	**02142**
(Address of principal executive offices)	**(Zip Code)**

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

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

Title of each class	Trading Symbol	Name of exchange on which registered
Common Stock, $0.0001 par value per share	SRPT	The Nasdaq Global Select Market

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

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

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

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

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

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

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date.

Common Stock with $0.0001 par value	**93,278,902**
(Class)	**(Outstanding as of July 28, 2023)**

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Item 1. Financial Statements

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited, in thousands, except share and per share amounts)

	As of June 30, 2023	As of December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 851,929	$ 966,777
Short-term investments	1,008,786	1,022,597
Accounts receivable	236,808	214,628
Inventory	226,876	203,968
Other current assets	148,215	149,891
Total current assets	2,472,614	2,557,861
Property and equipment, net	188,874	180,037
Right of use assets	134,728	64,954
Non-current inventory	166,635	162,545
Other non-current assets	163,039	162,969
Total assets	$ 3,125,890	$ 3,128,366
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 109,796	$ 95,875
Accrued expenses	326,877	418,996
Deferred revenue, current portion	44,989	89,244
Other current liabilities	16,992	15,489
Total current liabilities	498,654	619,604
Long-term debt	1,235,517	1,544,292
Lease liabilities, net of current portion	129,170	57,578
Deferred revenue, net of current portion	485,000	485,000
Contingent consideration	36,100	36,900
Other non-current liabilities	38	42
Total liabilities	2,384,479	2,743,416
Commitments and contingencies (Note 16)		
Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 198,000,000 shares authorized; 93,273,541 and 87,950,117 issued and outstanding at June 30, 2023, and December 31, 2022, respectively	9	9
Additional paid-in capital	5,193,388	4,296,841
Accumulated other comprehensive loss, net of tax	(1,055)	(1,664)
Accumulated deficit	(4,450,931)	(3,910,236)
Total stockholders' equity	741,411	384,950
Total liabilities and stockholders' equity	$ 3,125,890	$ 3,128,366

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited, in thousands, except per share amounts)

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2023	2022	2023	2022
Revenues:				
Products, net	$ 238,988	$ 211,237	$ 470,483	$ 400,062
Collaboration	22,250	22,250	44,255	44,255
Total revenues	261,238	233,487	514,738	444,317
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	34,124	37,795	69,141	69,238
Research and development	241,890	252,329	487,569	446,579
Selling, general and administrative	118,564	154,316	229,278	226,156
Amortization of in-licensed rights	179	179	357	357
Total cost and expenses	394,757	444,619	786,345	742,330
Operating loss	(133,519)	(211,132)	(271,607)	(298,013)
Other income (loss), net:				
Gain from sale of Priority Review Voucher	102,000	—	102,000	—
Loss on debt extinguishment	—	—	(387,329)	—
Other income (expense), net	16,934	(16,961)	29,641	(34,226)
Total other income (loss), net	118,934	(16,961)	(255,688)	(34,226)
Loss before income tax expense	(14,585)	(228,093)	(527,295)	(332,239)
Income tax expense	9,355	3,388	13,400	4,267
Net loss	(23,940)	(231,481)	(540,695)	(336,506)
Other comprehensive (loss) income:				
Unrealized (losses) gains on investments, net of tax	(636)	(2,179)	609	(2,465)
Total other comprehensive (loss) income	(636)	(2,179)	609	(2,465)
Comprehensive loss	$ (24,576)	$ (233,660)	$ (540,086)	$ (338,971)
Net loss per share - basic and diluted	$ (0.27)	$ (2.65)	$ (6.11)	$ (3.85)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	88,743	87,511	88,466	87,383

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(unaudited, in thousands)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
BALANCE AT DECEMBER 31, 2022	87,950	$ 9	$ 4,296,841	$ (1,664)	$ (3,910,236)	$ 384,950
Exercise of options for common stock	267	—	22,808	—	—	22,808
Vest of restricted stock units	390	—	—	—	—	—
Issuance of common stock for exchange of 2024 Notes	4,456	—	693,377	—	—	693,377
Partial settlement of capped call share options for 2024 Notes	—	—	80,645	—	—	80,645
Issuance of common stock under employee stock purchase plan	77	—	5,229	—	—	5,229
Stock-based compensation	—	—	41,250	—	—	41,250
Unrealized gains from available-for-sale securities, net of tax	—	—	—	1,245	—	1,245
Net loss	—	—	—	—	(516,755)	(516,755)
BALANCE AT MARCH 31, 2023	93,140	$ 9	$ 5,140,150	$ (419)	$ (4,426,991)	$ 712,749
Exercise of options for common stock	80	—	5,861	—	—	5,861
Vest of restricted stock units	54	—	—	—	—	—
Stock-based compensation	—	—	47,377	—	—	47,377
Unrealized losses from available-for-sale securities, net of tax	—	—	—	(636)	—	(636)
Net loss	—	—	—	—	(23,940)	(23,940)
BALANCE AT JUNE 30, 2023	93,274	$ 9	$ 5,193,388	$ (1,055)	(4,450,931)	$ 741,411

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
BALANCE AT DECEMBER 31, 2021	87,127	$ 9	$ 4,134,768	$ (20)	$ (3,206,748)	$ 928,009
Exercise of options for common stock	18	—	997	—	—	997
Vest of restricted stock units	289	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	62	—	3,993	—	—	3,993
Stock-based compensation	—	—	29,198	—	—	29,198
Unrealized losses from available-for-sale securities, net of tax	—	—	—	(286)	—	(286)
Net loss	—	—	—	—	(105,025)	(105,025)
BALANCE AT MARCH 31, 2022	87,496	$ 9	$ 4,168,956	$ (306)	$ (3,311,773)	$ 856,886
Exercise of options for common stock	11	—	339	—	—	339
Vest of restricted stock units	28	—	—	—	—	—
Stock-based compensation	—	—	102,892	—	—	102,892
Unrealized losses from available-for-sale securities, net of tax	—	—	—	(2,179)	—	(2,179)
Net loss	—	—	—	—	(231,481)	(231,481)
BALANCE AT JUNE 30, 2022	87,535	$ 9	$ 4,272,187	$ (2,485)	$ (3,543,254)	$ 726,457

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited, in thousands)

	For the Six Months Ended June 30,	
	2023	**2022**
Cash flows from operating activities:		
Net loss	$ (540,695)	$ (336,506)
Adjustments to reconcile net loss to cash flows from operating activities:		
Loss on debt extinguishment	387,329	—
Gain from sale of Priority Review Voucher	(102,000)	—
Depreciation and amortization	22,097	20,608
Reduction in the carrying amounts of the right of use assets	6,811	5,514
Non-cash interest expense	2,688	3,988
Stock-based compensation	88,627	132,090
Loss on disposal of assets	322	5,370
Accretion of investment discount, net	(20,245)	(1,459)
Other	(477)	468
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable	(22,180)	(50,864)
Net increase in inventory	(26,998)	(15,364)
Net decrease in other assets	11,015	30,921
Net decrease in deferred revenue	(44,255)	(44,255)
Net (decrease) increase in accounts payable, accrued expenses, lease liabilities and other liabilities	(93,669)	81,499
Net cash used in operating activities	(331,630)	(167,990)
Cash flows from investing activities:		
Proceeds from sale of Priority Review Voucher	102,000	—
Purchase of property and equipment	(27,394)	(14,629)
Purchase of available-for-sale securities	(829,799)	(1,137,602)
Maturity and sale of available-for-sale securities	864,458	77,151
Other	(139)	(718)
Net cash provided by (used in) investing activities	109,126	(1,075,798)
Cash flows from financing activities:		
Partial settlement of capped call share options for 2024 Notes	80,645	—
Proceeds from exercise of stock options and purchase of stock under the Employee Stock Purchase Program	33,898	5,329
Debt conversion costs for 2024 Notes	(6,887)	—
Net cash provided by financing activities	107,656	5,329
Decrease in cash, cash equivalents and restricted cash	(114,848)	(1,238,459)
Cash, cash equivalents and restricted cash:		
Beginning of period	985,801	2,125,523
End of period	$ 870,953	$ 887,064
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 851,929	$ 868,565
Restricted cash in other assets	19,024	18,499
Total cash, cash equivalents and restricted cash	$ 870,953	$ 887,064
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 7,817	$ 27,780
Supplemental schedule of non-cash investing activities and financing activities:		
Intangible assets and property and equipment included in accounts payable and accrued expenses	$ 20,928	$ 5,751
Lease liabilities arising from obtaining right of use assets	$ 75,875	$ 11,407
Common stock issued for exchange of 2024 Notes	$ 693,377	$ —
Lease liabilities terminated	$ —	$ 3,807

See accompanying notes to unaudited condensed consolidated financial statements.

1. ORGANIZATION AND NATURE OF BUSINESS

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

The Company's products in the U.S., EXONDYS 51 (eteplirsen) Injection (“EXONDYS 51”), VYONDYS 53 (golodirsen) Injection (“VYONDYS 53”), AMONDYS 45 (casimersen) Injection (“AMONDYS 45”) and ELEVIDYS, were granted accelerated approval by the U.S. Food and Drug Administration (the “FDA”) on September 19, 2016, December 12, 2019, February 25, 2021 and June 22, 2023, respectively. Indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 51, exon 53 and exon 45 skipping, respectively, EXONDYS 51, VYONDYS 53 and AMONDYS 45 use the Company’s phosphorodiamidate morpholino oligomer (“PMO”) chemistry and exon-skipping technology to skip exon 51, exon 53 and exon 45 of the dystrophin gene. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein. ELEVIDYS addresses the root genetic cause of Duchenne mutations in the dystrophin gene that result in the lack of dystrophin protein by delivering a gene that codes for a shortened form of dystrophin to muscle cells known as ELEVIDYS micro-dystrophin.

As of June 30, 2023, the Company had approximately $1,879.7 million of cash, cash equivalents, restricted cash and investments, consisting of $851.9 million of cash and cash equivalents, $1,008.8 million of short-term investments and $19.0 million of long-term restricted cash. The Company believes that its balance of cash, cash equivalents and investments as of the date of the issuance of this report is sufficient to fund its current operational plan for at least the next twelve months, though it may pursue additional cash resources through public or private debt and equity financings, seek funded research and development arrangements and additional government contracts and establish collaborations with or license its technology to other companies.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States (“U.S. GAAP”), reflect the accounts of Sarepta and its wholly-owned subsidiaries. All intercompany transactions between and among its consolidated subsidiaries have been eliminated. Management has determined that the Company operates in one segment: discovering, developing, manufacturing and delivering therapies to patients with rare diseases.

In the opinion of the Company’s management, all adjustments of a normal recurring nature necessary for a fair presentation have been reflected. Certain financial information that is normally included in annual financial statements prepared in accordance with U.S. GAAP, but that is not required for interim reporting purposes, has been omitted. These unaudited condensed consolidated financial statements should be read in conjunction with the consolidated financial statements and related notes for the year ended December 31, 2022 which are contained in the Company’s Annual Report on Form 10-K for the year ended December 31, 2022, filed with the U.S. Securities and Exchange Commission on February 28, 2023. The results for the three and six months ended June 30, 2023 are not necessarily indicative of the results to be expected for the full year.

Estimates and Uncertainties

The preparation of the unaudited condensed 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.

Concentration of Credit Risk

Financial instruments that potentially subject the Company to concentrations of credit risk consist of cash held at financial institutions, cash equivalents, investments and accounts receivable from customers. As of June 30, 2023, the Company’s cash was concentrated at three financial institutions, which potentially exposes the Company to credit risks. However, the Company does not

believe that there is significant risk of non-performance by the financial institutions. The Company also purchases commercial paper, government and government agency bonds, corporate bonds and certificates of deposit issued by highly rated corporations, financial institutions and governments and limits the amount of credit exposure to any one issuer. These amounts may at times exceed federally insured limits. The Company has not experienced any credit losses related to these financial instruments and does not believe to be exposed to any significant credit risk related to these instruments.

Please refer to *Note 7, Product Revenues, Net, Accounts Receivable and Reserves for Product Revenues* for discussion of the credit risk associated with accounts receivable from customers.

Significant Accounting Policies

For details about the Company's accounting policies, please read *Note 2, Summary of Significant Accounting Policies* of the Annual Report on Form 10-K for the year ended December 31, 2022.

There have not been any material changes to the Company's accounting policies through June 30, 2023.

3. LICENSE AND COLLABORATION AGREEMENTS

F. Hoffman-La Roche Ltd.

For the three and six months ended June 30, 2023 and 2022, the Company recognized $22.3 million and $44.3 million of collaboration revenue, respectively, associated with the license, collaboration and option agreement (the “Roche Agreement”) with F. Hoffman-La Roche Ltd. (“Roche”). As of June 30, 2023, the Company had total deferred revenue of $530.0 million associated with the Roche Agreement, of which $45.0 million is classified as current. The portion of deferred revenue related to the separate material rights for the options to acquire ex-U.S. rights to certain Duchenne-specific programs was $485.0 million as of June 30, 2023 and December 31, 2022.

The costs associated with co-development activities performed under the Roche Agreement are included in operating expenses, with any reimbursement of costs by Roche reflected as a reduction of such expenses when the related expense is incurred. For the three and six months ended June 30, 2023, costs reimbursable by Roche and reflected as a reduction to operating expenses were $28.2 million and $48.5 million, respectively. For the three and six months ended June 30, 2022, costs reimbursable by Roche and reflected as a reduction to operating expenses were $26.4 million and $44.1 million, respectively. As of June 30, 2023, there was $28.6 million of collaboration receivable included in other current assets.

Nationwide Children's Hospital

In December 2016, the Company entered into an exclusive option agreement with Nationwide Children’s Hospital (“Nationwide”) from which the Company obtained an exclusive right to acquire a worldwide license of the micro-dystrophin gene therapy technology for Duchenne and Becker muscular dystrophy. In October 2018, the Company exercised the option and entered into a license agreement with Nationwide, which granted the Company exclusive worldwide rights to develop, manufacture and commercialize a micro-dystrophin gene therapy product candidate. In connection with the FDA approval of ELEVIDYS in June 2023, the Company recorded a milestone payment of $10.0 million to Nationwide as an in-licensed right intangible asset in its unaudited condensed consolidated balance sheets as of June 30, 2023. The in-licensed right is being amortized on a straight-line basis over the remaining life of the relevant patents and has a carrying value of approximately $10.0 million as of June 30, 2023. Upon commercial sale of ELEVIDYS, the Company is expected to make low-single-digit royalty payments based on net sales which will be recorded by the Company as cost of sales.

Research and Option Agreements

The Company has research and option agreements with third parties in order to develop various technologies and biologics that may be used in the administration of the Company’s genetic therapeutics. The agreements generally provide for research services related to pre-clinical development programs and options to license the technology for clinical development. Prior to the options under these agreements being executed, the Company may be required to make up to $39.8 million in research milestone payments. Under these agreements, there are $221.3 million in potential option payments to be made by the Company upon the determination to exercise the options. Additionally, if the options for each agreement are exercised and additional license agreements are executed, the Company would incur additional contingent obligations and may be required to make development, regulatory, and sales milestone payments and royalty payments based on the net sales of the developed products upon commercialization. In April 2023, the Company exercised one such option and recognized $7.5 million of up-front payment as research and development expense in the accompanying unaudited condensed consolidated statements of operations and comprehensive loss for the three and six months ended June 30, 2023. For the three and six months ended June 30, 2022, the Company recognized $6.0 million of research, option and milestone expense. As of June 30, 2023, no additional research milestone payments became probable of occurring.

Milestone Obligations

The Company has license and collaboration agreements in place for which it could be obligated to pay, in addition to the payment of up-front fees upon execution of the agreements, certain milestone payments as a product candidate proceeds from the submission of an investigational new drug application through approval for commercial sale and beyond. As of June 30, 2023, the Company may be obligated to make up to $3.2 billion of future development, regulatory, commercial and up-front royalty payments associated with its collaboration and license agreements. These obligations exclude potential future option and milestone payments for options that have yet to be exercised within agreements entered into by the Company as of June 30, 2023, which are discussed above. For the three and six months ended June 30, 2023, the Company recognized up-front, development milestone and other expenses of $7.6 million and $8.1 million, respectively, as research and development expense in the accompanying unaudited condensed consolidated statements of operations and comprehensive loss. For the three and six months ended June 30, 2022, the Company recognized up-front and development milestone expenses of approximately $8.8 million as research and development expense in the accompanying unaudited condensed consolidated statements of operations and comprehensive loss.

4. GAIN FROM SALE OF PRIORITY REVIEW VOUCHER

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

5. FAIR VALUE MEASUREMENTS

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

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

During the six and twelve months ended June 30, 2023 and December 31, 2022, there were no transfers into or out of Level 3. The tables below present information about the Company’s financial assets and liabilities that are measured and carried at fair value and indicate the level within the fair value hierarchy of the valuation techniques it utilizes to determine such fair value:

	Fair Value Measurement as of June 30, 2023			
	Total	Level 1	Level 2	Level 3
		(in thousands)		
Assets				
Money market funds	$ 404,863	$ 404,863	$ —	$ —
Commercial paper	169,866	—	169,866	—
Government and government agency bonds	859,532	—	859,532	—
Corporate bonds	21,207	—	21,207	—
Strategic investments	31,000	—	—	31,000
Certificates of deposit	7,961	—	7,961	—
Total assets	$ 1,494,429	$ 404,863	$ 1,058,566	$ 31,000
Liabilities				
Contingent consideration	$ 36,100	$ —	$ —	$ 36,100
Total liabilities	$ 36,100	$ —	$ —	$ 36,100

	Fair Value Measurement as of December 31, 2022			
	Total	Level 1	Level 2	Level 3
		(in thousands)		
Assets				
Money market funds	$ 467,553	$ 467,553	$ —	$ —
Commercial paper	211,369	—	211,369	—
Government and government agency bonds	807,540	—	807,540	—
Corporate bonds	125,741	—	125,741	—
Strategic investments	31,321	321	—	31,000
Certificates of deposit	42,745	—	42,745	—
Total assets	$ 1,686,269	$ 467,874	$ 1,187,395	$ 31,000
Liabilities				
Contingent consideration	$ 36,900	$ —	$ —	$ 36,900
Total liabilities	$ 36,900	$ —	$ —	$ 36,900

The Company’s assets with a fair value categorized as Level 1 within the fair value hierarchy primarily include money market funds.

The Company's assets with a fair value categorized as Level 2 within the fair value hierarchy consist of commercial paper, government and government agency bonds, corporate bonds and certificates of deposit. These assets have been initially valued at the transaction price and subsequently valued at the end of each reporting period utilizing third-party pricing services. The Company uses observable market inputs to determine value, which primarily consist of reportable trades. Certain of the short-term investments with maturities of less than three months at the date of acquisition are presented as cash equivalents on the unaudited condensed consolidated balance sheets as of June 30, 2023.

The Company’s assets with a fair value categorized as Level 3 within the fair value hierarchy consist of a strategic investment in Series A preferred stock of Lacerta Therapeutics, Inc. (“Lacerta”) and strategic investments in two other private companies. For more information related to Lacerta, please read *Note 3, License and Collaboration Agreements* to the financial statements included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2022. The fair value of the Lacerta investment was initially based on a cost approach corroborated by the Black-Scholes-Merton option-pricing model. The most significant assumptions in the option pricing model include historical volatility of similar public companies, estimated term through Lacerta’s potential exit and a risk-free rate based on certain U.S. Treasury rates. The investments in the other two private companies are recorded at fair value at the time of purchase as measured by their respective investment cost. At the end of each reporting period, the fair value of the Company's strategic investments will be adjusted if the issuers were to issue similar or identical securities or when there is a triggering event for impairment. There were no valuation measurement events related to the fair value of the Company's Level 3 strategic investments during the six months ended June 30, 2023 or 2022, as no impairment indicators were identified nor were similar securities issued.

The Company’s contingent consideration liability with a fair value categorized as Level 3 within the fair value hierarchy relates to the regulatory-related contingent payments to Myonexus Therapeutics, Inc. (“Myonexus”) selling shareholders as well as to two academic institutions under separate license agreements that meet the definition of a derivative. For more information related to Myonexus, please read *Note 3, License and Collaboration Agreements* to the financial statements included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2022. The contingent consideration liability was estimated using an income approach based on the probability-weighted expected cash flows that incorporated industry-based probability adjusted assumptions relating to the achievement of the milestone and thus the likelihood of making the payments. This fair value measurement was based upon significant inputs not observable in the market and therefore represented a Level 3 measurement. Significant changes which increase or decrease the probabilities of achieving the milestone, or shorten or lengthen the time required to achieve the milestone, would result in a corresponding increase or decrease in the fair value of the liability. At the end of each reporting period, the fair value is adjusted to reflect the most current assumptions through earnings.

A decrease of $0.8 million was recorded during the three and six months ended June 30, 2023 to account for the change in fair value of the Company's contingent consideration liabilities. This change, which is recorded through earnings, was a result of the termination of a license agreement with an academic institution that had met the definition of a derivative. As of June 30, 2023, the remaining contingent consideration was recorded as a non-current liability on the Company's unaudited condensed consolidated balance sheets.

The carrying amounts reported in the unaudited condensed consolidated balance sheets for cash and cash equivalents, accounts receivable and accounts payable approximated fair value because of the immediate or short-term maturity of these financial instruments. For fair value information related to the Company's debt facilities, please read *Note 11, Indebtedness.*

6. CASH, CASH EQUIVALENTS AND MARKETABLE SECURITIES

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

	As of June 30, 2023	As of December 31, 2022
	(in thousands)	
Money market funds	$ 404,863	$ 467,553
Corporate bonds	49,780	3,157
Government and government agency bonds	—	128,451
Commercial paper	—	33,190
Total	$ 454,643	$ 632,351

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 both June 30, 2023 and December 31, 2022 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 June 30, 2023			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
		(in thousands)		
Cash and money market funds	$ 802,149	$ —	$ —	$ 802,149
Commercial paper	169,866	—	—	169,866
Government and government agency bonds	860,546	65	(1,079)	859,532
Corporate bonds	21,221	5	(19)	21,207
Certificates of deposit	7,961	—	—	7,961
Total cash, cash equivalents and investments	$ 1,861,743	$ 70	$ (1,098)	$ 1,860,715
As reported:				
Cash and cash equivalents	$ 851,917	$ 12	$ —	$ 851,929
Short-term investments	1,009,826	58	(1,098)	1,008,786
Total cash, cash equivalents and investments	$ 1,861,743	$ 70	$ (1,098)	$ 1,860,715

	As of December 31, 2022			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
		(in thousands)		
Cash and money market funds	$ 801,979	$ —	$ —	$ 801,979
Commercial paper	211,369	—	—	211,369
Government and government agency bonds	808,904	178	(1,542)	807,540
Corporate bonds	126,014	9	(282)	125,741
Certificates of deposit	42,745	—	—	42,745
Total cash, cash equivalents and investments	$ 1,991,011	$ 187	$ (1,824)	$ 1,989,374
As reported:				
Cash and cash equivalents	$ 966,768	$ 9	$ —	$ 966,777
Short-term investments	1,024,243	178	(1,824)	1,022,597
Total cash, cash equivalents and investments	$ 1,991,011	$ 187	$ (1,824)	$ 1,989,374

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

For the three months ended June 30, 2023 and 2022, the Company recorded $239.0 million and $211.2 million, respectively, of product revenues, net. For the six months ended June 30, 2023 and 2022, the Company recorded $470.5 and $400.1 million, respectively, of products revenues, net. Three individual customers accounted for 47%, 33% and 7% of product revenues, net, for the three months ended June 30, 2023 and 48%, 32% and 7% for the six months ended June 30, 2023. Three individual customers accounted for 50%, 32% and 7% of net product revenues for the three months ended June 30, 2022 and 49%, 34% and 7% of net product revenues for the six months ended June 30, 2022. The Company considers there to be revenue concentration risks for regions where net product revenues exceed 10% of consolidated net product revenues. The concentration of the Company's net product revenues within a particular country may have a material adverse effect on the Company's revenues and results of operations if sales in the respective regions experience difficulties. For the three months ended June 30, 2023, net product revenues totaled $203.9 million and $35.1 million within the United States and the rest of the world, respectively. For the six months ended June 30, 2023, net product revenues totaled $404.4 million and $66.1 million within the United States and the rest of world, respectively. For the three months ended June 30, 2022, net product revenues totaled $187.6 million and $23.6 million within the United States and the rest of the world, respectively. For the six months ended June 30, 2022, net product revenues totaled $360.0 million and $40.1 million within the United States and the rest of the world, respectively. No individual rest of world country exceeded 10% of total net product revenues for the three and six months ended June 30, 2023 and 2022.

As of June 30, 2023 and December 31, 2022, the Company's accounts receivable were $236.8 million and $214.6 million, respectively, both of which were primarily related to product sales receivable, net of discounts and allowances. As of June 30, 2023, the majority of the Company's accounts receivable arose from product sales in the U.S. and all customers have standard payment terms that generally require payment within 60 to 91 days. Outside of the U.S., the majority of the Company's customers have payment terms ranging between 60 and 150 days. Three individual customers accounted for 35%, 34% and 12% of accounts receivable from product sales as of June 30, 2023 and 36%, 35% and 12% of accounts receivable from product sales as of December 31, 2022. As of June 30, 2023, the Company believes that such customers are of high credit quality and has not experienced any material credit losses related to such customers.

The following tables summarize an analysis of the change in reserves for discounts and allowances for each of the periods indicated:

	Chargebacks	Rebates	Prompt Pay	Other Accruals	Total
			(in thousands)		
Balance, as of December 31, 2022	$ 417	$ 67,493	$ 3,343	$ 23,445	$ 94,698
Provision	6,287	60,824	7,228	32,220	106,559
Adjustments relating to prior years	—	(3,437)	—	—	(3,437)
Payments/credits	(6,434)	(45,592)	(6,976)	(24,820)	(83,822)
Balance, as of June 30, 2023	$ 270	$ 79,288	$ 3,595	$ 30,845	$ 113,998

	Chargebacks	Rebates	Prompt Pay	Other Accruals	Total
			(in thousands)		
Balance, as of December 31, 2021	$ 799	$ 60,506	$ 2,798	$ 6,363	$ 70,466
Provision	5,642	52,589	6,192	19,386	83,809
Adjustments relating to prior years	—	(2,228)	—	30	(2,198)
Payments/credits	(6,232)	(43,293)	(5,639)	(11,460)	(66,624)
Balance, as of June 30, 2022	$ 209	$ 67,574	$ 3,351	$ 14,319	$ 85,453

The following table summarizes the total reserves above included in the Company's unaudited condensed consolidated balance sheets for each of the periods indicated:

	As of June 30, 2023	As of December 31, 2022
	(in thousands)	
Reduction to accounts receivable	$ 33,574	$ 25,914
Component of accrued expenses	80,424	68,784
Total reserves	$ 113,998	$ 94,698

8. INVENTORY

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

	As of June 30, 2023	As of December 31, 2022
	(in thousands)	
Raw materials	$ 102,838	$ 59,181
Work in progress	243,061	269,185
Finished goods	47,612	38,147
Total inventory	$ 393,511	$ 366,513

Non-current inventory consists of raw materials and work in progress and is anticipated to be consumed beyond the Company's normal operating cycle.

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

	As of June 30, 2023	As of December 31, 2022
	(in thousands)	
Balance sheet classification		
Inventory	$ 226,876	$ 203,968
Non-current inventory	166,635	162,545
Total inventory	$ 393,511	$ 366,513

9. OTHER ASSETS

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

	As of June 30, 2023	As of December 31, 2022
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 79,007	$ 66,455
Collaboration receivable	28,635	41,758
Prepaid maintenance services	11,777	9,815
Prepaid clinical and pre-clinical expenses	7,127	11,237
Prepaid insurance	3,139	3,717
Prepaid commercial expenses	2,854	2,947
Prepaid research expenses	2,502	1,927
Interest receivable	1,715	3,311
Other	11,459	8,724
Total other current assets	$ 148,215	$ 149,891

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

	As of June 30, 2023	As of December 31, 2022
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 83,479	$ 97,409
Strategic investments	31,000	31,321
Restricted cash	19,024	19,024
Intangible assets, net	18,018	7,578
Prepaid clinical expenses	2,196	2,150
Other	9,322	5,487
Total other non-current assets	$ 163,039	$ 162,969

10. ACCRUED EXPENSES

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

	As of June 30, 2023	As of December 31, 2022
	(in thousands)	
Accrued contract manufacturing costs	$ 82,734	$ 202,173
Product revenue related reserves	80,424	68,784
Accrued employee compensation costs	44,906	65,946
Accrued clinical and pre-clinical costs	39,786	28,884
Accrued income taxes	20,815	12,521
Accrued professional fees	19,984	12,061
Accrued milestone and license costs	11,200	7,702
Accrued royalties	8,583	8,636
Accrued fixed assets	6,027	984
Accrued research costs	2,627	1,629
Other	9,791	9,676
Total accrued expenses	$ 326,877	$ 418,996

11. INDEBTEDNESS

2024 Convertible Notes and 2017 Capped Call Transactions

On November 14, 2017, the Company issued $570.0 million aggregate principal amount of senior convertible notes due on November 15, 2024 (the "2024 Notes") and, simultaneously, entered into capped call transactions with counterparties intended to minimize the impact of potential dilution upon conversion of the 2024 Notes (the "2017 Capped Calls").

On September 14, 2022, the Company entered into separate, privately negotiated transactions to repurchase a portion of the outstanding 2024 Notes. The holders exchanged $150.6 million in aggregate principal value of 2024 Notes held by them for an aggregate payment of $248.6 million for full settlement of the principal value and accrued interest on such date. As a result of the repurchases, the Company entered into agreements with the 2017 Capped Calls counterparties to terminate a corresponding portion of the 2017 Capped Calls and received $26.3 million in cash.

On March 2, 2023, the Company entered into separate, privately negotiated exchange agreements with certain holders of the outstanding 2024 Notes (the "Exchange Agreements"). The Exchange Agreements resulted in an exchange of $313.5 million in aggregate principal value of the 2024 Notes for shares of the Company's common stock (the "2024 Notes Exchange"), which closed on March 7, 2023 (the "Exchange Date"). In connection with the 2024 Notes Exchange, the Company issued approximately 4.5 million shares of the Company's common stock representing an agreed upon contractual exchange rate under each of the Exchange Agreements. The fair value of these shares issued was approximately $693.4 million. The Company also incurred approximately $6.9 million in third-party debt conversion costs. The exchange was not pursuant to the conversion privileges included in the terms of the debt at issuance and therefore was accounted for as a debt extinguishment.

The Company accounted for the debt extinguishment by recognizing the difference between the fair value of the shares of common stock transferred on the Exchange Date and the net carrying amount of the extinguished debt as a loss on debt extinguishment. Accordingly, on the Exchange Date, the Company: (i) reduced the carrying value of the 2024 Notes by $311.5 million, (ii) eliminated accrued interest of $1.5 million, and (iii) recorded a loss on debt extinguishment of $387.3 million, inclusive of the $6.9 million in third-party debt conversion costs, which is included in the unaudited condensed consolidated statement of operations and comprehensive loss. The outstanding principal balance of the 2024 Notes as of June 30, 2023 is approximately $105.8 million, which is convertible into approximately 1.4 million shares of Company common stock.

As a result of the exchange, the Company entered into agreements with the counterparties to the 2017 Capped Calls to terminate a portion of the 2017 Capped Calls in a notional amount corresponding to the principal amount of the 2024 Notes exchanged through the 2024 Notes Exchange and received approximately $80.6 million in cash from the counterparties, which was included in additional paid-in capital within the unaudited condensed consolidated balance sheets as of June 30, 2023.

For additional details about the 2024 Notes, please read *Note 13, Indebtedness* to the financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2022.

Total Debt Obligations

As of June 30, 2023 and December 31, 2022, the Company recorded approximately $1,235.5 million and $1,544.3 million as long-term debt on the unaudited condensed consolidated balance sheets, respectively.

The following table summarizes the Company's debt facilities for each of the periods indicated:

	As of June 30, 2023	As of December 31, 2022
	(in thousands)	
Principal amount of the 2024 Notes	$ 105,847	$ 419,371
Principal amount of the 2027 Notes	1,150,000	1,150,000
Unamortized discount - debt issuance costs of 2024 Notes	(567)	(3,059)
Unamortized discount - debt issuance costs of 2027 Notes	(19,763)	(22,020)
Total carrying value of debt facilities	$ 1,235,517	$ 1,544,292
Fair value of 2024 Notes	$ 202,287	$ 765,046
Fair value of 2027 Notes	1,247,693	1,308,482
Total fair value of debt facilities	$ 1,449,980	$ 2,073,528

For the three months ended June 30, 2023 and 2022, contractual interest expense from debt facilities was $5.2 million and $16.0 million, inclusive of $1.2 million and $2.0 million of amortization of debt discounts, respectively. For the six months ended June 30, 2023 and 2022, contractual interest expense from debt facilities was $11.5 million and $31.8 million, inclusive of $2.7 million and $4.0 million of amortization of debt discounts, respectively. The fair value of the 1.25% convertible senior notes due on September 15, 2027 ("2027 Notes") and the 2024 Notes is based on open market trades and is classified as Level 1 in the fair value hierarchy.

The following table summarizes the total principal payments due under the Company's debt arrangements:

	As of June 30, 2023
	(in thousands)
2023 (July-December)	$ —
2024	105,847
2025	—
2026	—
2027	1,150,000
Thereafter	—
Total payments	$ 1,255,847

The aggregate annual maturities of long-term debt principal and contractual interest during the remainder of 2023, the years ending December 31, 2024, 2025, 2026, and 2027 are $8.0 million, $121.8 million, $14.4 million, $14.4 million and $1,164.3 million, respectively.

12. STOCK-BASED COMPENSATION

The following table summarizes the Company's stock awards granted for each of the periods indicated:

	For the Three Months Ended June 30,				For the Six Months Ended June 30,			
	2023		2022		2023		2022	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options	54,575	$ 66.82	207,583	$ 43.40	1,084,924	$ 72.50	1,499,120	$ 46.49
Restricted stock units*	33,752	$ 121.27	147,075	$ 72.99	1,109,663	$ 153.89	859,510	$ 78.78

*Included in restricted stock units ("RSUs") for the three and six months ended June 30, 2023 and 2022 are 502,225 and 38,500 shares, respectively, with performance conditions ("PSUs") which are related to regulatory approval of certain of the Company's product candidates. As a result of the regulatory approval of ELEVIDYS in June 2023, the Compensation Committee of the Company's Board of Directors determined that one of the performance conditions was met, resulting in the recognition of stock-based compensation expense of $7.4 million associated with 394,975 PSUs with performance conditions during the three and six months ended June 30, 2023. Vesting of the 394,975 PSUs with performance conditions achieved is contingent on the fulfillment of remaining service conditions. The remaining expense associated with the 394,975 PSUs with performance conditions achieved is $54.2 million and will be recognized over approximately the next 1.7 years. If the performance milestones are met within the required time frame for the remaining PSUs, the Company may recognize up to $22.4

million of stock-based compensation expense. Stock options and the remaining RSUs granted during the periods presented in the table have only service-based criteria and vest over four years.

Grant Modification

In June 2017, the Company granted its Chief Executive Officer 3,300,000 options with service and market conditions which were subject to a five-year cliff vesting schedule. On April 19, 2022 (the "Effective Date"), the Company entered into an agreement with its Chief Executive Officer to modify the vesting conditions of the options. Under the agreement, one-third of the options vested (the "Vested Tranche") on the Effective Date with no required service or market conditions. Subject to the Chief Executive Officer's continued service through each applicable vesting date and the compound annual growth rate of the Company's common stock exceeding that of the Nasdaq Biotech Index in varying percentages, the remaining two-thirds of the options (the "Unvested Tranche") shall vest in varying increments at any time between the Effective Date and June 26, 2025 (the "Measurement Period") when (and if) the average of the closing price of the Company's common stock during any consecutive 20 trading day period during the Measurement Period reaches certain pre-determined target stock prices.

The Unvested Tranche represents awards with market conditions only. Both the pre- and post-modification fair values for the Unvested Tranche are determined by a lattice model with Monte Carlo simulations. The incremental compensation costs related to varying increments of the Unvested Tranche will be recognized as stock-based compensation expense over their respective derived service periods, an output from the Monte Carlo simulation, and will be fully recognized over a 1.3 year period from the Effective Date.

During the six months ended June 30, 2023, 550,110 options relating to the Unvested Tranche met the conditions for vesting as the average closing price of the Company's common stock exceeded $128.65 during 20 consecutive trading days in March 2023 and the compound annual growth rate of the Company's common stock exceeded that of the Nasdaq Biotech Index by greater than 5%. For the three and six months ended June 30, 2023, the Company recorded $4.4 million and $12.8 million of stock-based compensation expense in total related to the Chief Executive Officer's awards, respectively. As of June 30, 2023, the Company is expected to recognize incremental compensation cost of $0.6 million over approximately the next one month associated with the Unvested Tranche.

Stock-based Compensation Expense

For the three months ended June 30, 2023 and 2022, total stock-based compensation expense was $47.4 million and $102.9 million, respectively. For the six months ended June 30, 2023 and 2022, total stock-based compensation expense was $88.6 million and $132.1 million, respectively. The following table summarizes stock-based compensation expense by function included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2023	2022	2023	2022
	(in thousands)			
Research and development	$ 21,577	$ 14,467	$ 37,990	$ 27,535
Selling, general and administrative	25,800	88,425	50,637	104,555
Total stock-based compensation expense	$ 47,377	$ 102,892	$ 88,627	$ 132,090

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

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2023	2022	2023	2022
	(in thousands)			
Stock options	$ 21,135	$ 89,155	$ 44,965	$ 105,561
Restricted stock units	25,037	12,378	41,177	23,743
Employee stock purchase plan	1,205	1,359	2,485	2,786
Total stock-based compensation expense	$ 47,377	$ 102,892	$ 88,627	$ 132,090

13. OTHER INCOME (LOSS), NET

The following table summarizes other income (loss), net for each of the periods indicated:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2023	2022	2023	2022
	(in thousands)			
Accretion of investment discount, net	$ 12,786	$ 1,331	$ 22,825	$ 1,373
Interest income	8,418	2,409	17,694	2,582
Gain on contingent consideration	800	—	800	—
Interest expense	(5,224)	(16,028)	(11,547)	(31,824)
Other, net	154	(4,673)	(131)	(6,357)
Other income (expense), net	$ 16,934	$ (16,961)	$ 29,641	$ (34,226)
Gain from sale of Priority Review Voucher	102,000	—	102,000	—
Loss on debt extinguishment	—	—	(387,329)	—
Total other income (loss), net	$ 118,934	$ (16,961)	$ (255,688)	$ (34,226)

14. LEASES

The Company has real estate operating leases in Cambridge, Andover, Burlington and Bedford, Massachusetts, Dublin and Columbus, Ohio, and Durham, North Carolina that provide for scheduled annual rent increases over the lease term. The Company has also identified leases embedded in certain of its manufacturing and supply agreements as the Company determined that it controls the use of the facilities and related equipment therein. For more information related to manufacturing and supply agreements with Catalent, Inc. (“Catalent”), please refer to *Note 21, Commitments and Contingencies* of the Company's Annual Report on Form 10-K for the year ended December 31, 2022.

Bedford, Massachusetts

On April 22, 2022, the Company entered into a lease agreement (the “Bedford Lease”) for 288,000 square feet of to-be-constructed research and development and manufacturing space in Bedford, Massachusetts. The term of the Bedford Lease commences upon the landlord’s completion of the initial construction of the core and shell of the building, at which time the Company will obtain control of the premises and commence internal construction activities. The Company has two options to extend the lease for a period of ten years each, exercisable under certain conditions and at a market rate determined in accordance with the lease agreement.

In May 2022, in connection with the execution of the Bedford Lease, the Company issued a letter of credit collateralized by cash deposits of approximately $8.4 million, which was included in the other non-current assets of the Company’s unaudited condensed consolidated balance sheets. Such letter of credit shall be reduced to approximately $5.6 million at the commencement of the fourth rent year, provided certain conditions set forth in the Bedford Lease are satisfied.

Undiscounted minimum rent payments due over the term of the lease aggregate to $307.4 million. Additionally, the Company is responsible for reimbursing the landlord for the Company’s share of the property’s operating expenses and property taxes. The Bedford Lease also provides for a tenant improvement allowance from the landlord of up to $72.0 million to be used towards costs incurred by the Company in the design and construction of the premises.

The Bedford Lease commenced in May 2023 as the Company obtained control of the premises (the “Bedford Lease Commencement”). As a result, the Company recorded a lease liability and right-of-use asset of $76.5 million and $72.0 million, respectively, on its unaudited condensed consolidated balance sheets as of June 30, 2023. The Company recorded the $72.0 million tenant improvement allowance as a reduction to right-of-use assets and lease liabilities at the date of the Bedford Lease Commencement. Tenant improvement costs incurred by the Company that had been reimbursed by the landlord totaled $2.0 million as of June 30, 2023 and are recorded as an increase to lease liabilities within the Company's unaudited condensed consolidated balance sheets.

The initial gross ROU assets and lease liabilities recorded were based on the present value of estimated future payments associated with the Bedford Lease at a discount rate of 9.5%, representing the rate at which the Company could borrow on a collateralized basis the amount of the lease payments in a similar term. The remaining lease term for the Bedford Lease is 15.5 years. For the three and six months ended June 30, 2023, the Company had no operating lease costs or variable lease costs related to the Bedford Lease.

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 Three Months Ended June 30,		For the Six Months Ended June 30,	
	2023	2022	2023	2022
	(in thousands, except per share amounts)		(in thousands, except per share amounts)	
Net loss	$ (23,940)	$ (231,481)	$ (540,695)	$ (336,506)
Weighted-average common shares outstanding - basic	88,743	87,511	88,466	87,383
Effect of dilutive securities*	—	—	—	—
Weighted-average common shares outstanding - diluted	88,743	87,511	88,466	87,383
Net loss per share - basic and diluted	$ (0.27)	$ (2.65)	$ (6.11)	$ (3.85)

* For the three and six months ended June 30, 2023 and 2022, stock options, RSUs and employee stock purchase plan to purchase of approximately 12.3 million and 11.3 million shares of common stock, respectively, were excluded from the diluted net loss per share calculation as their effect would have been anti-dilutive. The Company accounts for the effect of its 2027 Notes and 2024 Notes on diluted net earnings per share ("EPS") using the if-converted method as this obligation may be settled in cash or shares at the Company's option. The effect of potential share settlement is included in the diluted EPS calculation if the effect is more dilutive. During the three and six months ended June 30, 2023, the inclusion of the potential share settlement of the 2027 Notes was anti-dilutive. During the three and six months ended June 30, 2023 and 2022 the inclusion of the potential share settlement of the 2024 Notes was anti-dilutive. Accordingly, the potential conversion of approximately 1.4 million and 7.8 million shares related to the 2024 Notes has been excluded from the computation of diluted net loss per share for the three and six months ended June 30, 2023 and 2022, respectively, and the potential conversion of approximately 8.1 million shares related to the 2027 Notes has been excluded from the computation of diluted net loss per share for the three and six months ended June 30, 2023.

16. COMMITMENTS AND CONTINGENCIES

Manufacturing Obligations

The following table summarizes the aggregate non-cancelable contractual obligations arising from the Company's manufacturing obligations:

	As of June 30, 2023
	(in thousands)
2023 (July-December)	$ 489,561
2024	416,331
2025	146,010
2026	80,110
2027	77,497
Thereafter	72,244
Total manufacturing commitments*	$ 1,281,753

* Total manufacturing commitments includes the Catalent manufacturing and supply agreement, for which the Company has right of use assets and lease liabilities recorded on the unaudited condensed consolidated balance sheets as of June 30, 2023. For more information, please read *Note 21, Commitments and Contingencies* to the financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2022.

Thermo Fisher Scientific, Inc.

The Company entered into a development, commercial manufacturing, and supply agreement in June 2018 and, subsequently, entered into the first, second and third amendments in May 2019, July 2020 and October 2021, respectively, with Brammer Bio MA, LLC, an affiliate of Thermo Fisher Scientific, Inc. ("Thermo") (collectively, the "Thermo Agreement").

In March 2023, the Company executed a fourth amendment (the "Amendment") that modified the terms of the Thermo Agreement. The Amendment removed the previous minimum batch purchase commitment of $54.7 million per annum and associated fee for the remaining term of the Thermo Agreement. In connection with the elimination of such commitment and fee, the Amendment implemented a fee of up to $60.0 million, to be paid in three installments of $20.0 million each by March 1, 2024, December 31, 2024 and December 31, 2025, respectively, unless waived in part as described below. The Company will recognize the first $20.0 million installment due March 1, 2024 as a nonrefundable advance payment over the term of the agreement as the Company believes it will receive future benefit from this contract. As the Company has yet to obtain regulatory approval to produce commercial supply of ELEVIDYS at Thermo manufacturing facilities as of June 30, 2023, it recognized approximately $0.9 million as research and development expense during the three and six months ended June 30, 2023 related to this nonrefundable advanced payment.

The second and third payment installments, which are associated with the years ending December 31, 2024 and 2025, will be waived if the Company meets certain minimum purchase thresholds under the Amendment. As of June 30, 2023, the Company believes it is probable that the minimum purchase thresholds will be met in the normal course of business throughout the term of the agreement and, therefore, no liabilities were recorded related to the second or third payment installments.

For more information related to Thermo, please read *Note 21, Commitments and Contingencies* to the financial statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2022.

Litigation

In the normal course of business, the Company from time to time is named as a party to various legal claims, actions and complaints, which have included or may include matters involving securities, employment, intellectual property, arising from the use of therapeutics utilizing its technology, or others. We record a loss contingency reserve for a legal proceeding when we consider the potential loss probable and we can reasonably estimate the amount of the loss or determine a probable range of loss. We provide disclosure when we consider a loss reasonably possible or when we determine that a loss in excess of a reserve is reasonably possible. We provide an estimate of such reasonably possible losses or an aggregate range of such reasonably possible losses, unless we believe that such an estimate cannot be made. The Company has not recorded any material accruals for loss contingencies and in management's opinion no material range of loss is estimable for the matters described below as of June 30, 2023.

On September 15, 2020, REGENXBIO INC. ("Regenx") and the Trustees of the University of Pennsylvania ("U-Penn") filed a lawsuit against the Company and Sarepta Therapeutics Three, LLC, in the U.S. District Court for the District of Delaware. The plaintiffs assert patent infringement of U.S. Patent No. 10,526,617 ("the '617 Patent") under 35 U.S.C.§§ 271(a)-(c) based on Sarepta's alleged direct or indirect manufacture and use of the patented cultured host cell technology allegedly used to make adeno-associated virus ("AAV") gene therapy products, including SRP-9001 (approved June 22, 2023 in the U.S. as ELEVIDYS®). Specifically, the Complaint essentially includes the allegation that Sarepta's use, and the use by its contract manufacturers on its behalf, of a host cell containing a recombinant acid molecule that encodes a capsid protein having at least 95% amino acid identity to AAVrh10 infringes the '617 Patent asserted by Regenx. Plaintiffs seek injunctive relief, a judgment of infringement and willful infringement, an unspecified amount of damages that is no less than a reasonable royalty (treble damages), attorneys' fees and costs, and such other relief as the court deems just and proper. On January 4, 2022, the Court denied Sarepta's motion to dismiss the case pursuant to Federal Rule of Civil Procedure 12(b)(6) based on the Safe Harbor provision of non-infringement contained in 35 U.S.C. § 271(e)(1). Sarepta answered the Complaint on January 18, 2022, and a case schedule has been set with a trial commencing on January 29, 2024.

On June 20, 2023, Regenx and U-Penn commenced a second patent infringement lawsuit against Sarepta and its contract manufacturer, Catalent asserting patent alleged infringement of U. S. Patent No. 11,680,274 ("the '274 Patent"). In the second lawsuit, Regenx and U-Penn allege that Sarepta and Catalent's manufacture, use and commercial launch of ELEVIDYS® (formerly/also known as SRP-9001) infringe the '274 Patent. Responsive pleadings are due on August 10, 2023. No case schedule has been set.

On July 13, 2021, Nippon Shinyaku Co., Ltd. ("Nippon Shinyaku" or "NS") filed a lawsuit against the Company in the U.S. District Court for the District of Delaware. NS asserts a claim for breach of contract arising from Sarepta filing seven petitions for Inter Partes Review ("IPR Petitions") with the Patent Trial and Appeal Board at the USPTO in which Sarepta sought to invalidate certain NS patents concerning exon 53 skipping technology (U.S. Patent Nos. 9,708,361, 10,385,092, 10,407,461, 10,487,106, 10,647,741, 10,662,217, and 10,683,322, respectively, and collectively the "NS Patents"). In addition, NS asserts claims for patent infringement and willful infringement of each of the NS Patents allegedly arising from Sarepta's activities, including the sale of, its exon 53 skipping product, VYONDYS 53 (golodirsen). NS further seeks a determination of non-infringement by NS alleged to arise from NS's activities, including the sale of, its exon 53 skipping product, Viltepso (viltolarsen) and invalidity of certain patents licensed to the Company from University of Western Australia ("UWA") (U.S. Patent Nos. 9,994,851, 10,227,590, and 10,266,827, collectively the "UWA Patents"). NS is seeking legal fees and costs, an unspecified amount of monetary relief (treble damages) attributed to Sarepta's alleged infringement, and such other relief as the court deems just and proper. In January 2022, the PTAB granted institution of all claims of all NS Patents in response to Sarepta's IPR Petitions and determined that Sarepta has demonstrated a reasonable likelihood of success in proving that the NS Patents are unpatentable. NS filed a motion for preliminary injunction solely

seeking Sarepta's withdrawal of the IPR Petitions, which was ultimately granted after the U.S. Court of Appeals for the Federal Circuit reversed and remanded to the district court on February 8, 2022. Sarepta subsequently withdrew the IPRs, which were terminated on June 14, 2022. On December 27, 2021, the district court partially granted and denied the motion to dismiss by Sarepta and ordered NS to file a Second Amended Complaint ("SAC"), which it did on January 14, 2022. In the SAC, NS maintains all claims of the original complaint of July 13, 2021, except a determination of non-infringement of the UWA Patents. On January 28, 2022, Sarepta filed its answer to the SAC, with defenses and counterclaims against NS and NS Pharma Inc. that include infringement of the UWA Patents allegedly arising from their activities concerning, including the sale of, its exon 53 skipping product, Viltepso (viltolarsen) and breach of contract. Sarepta is also seeking a determination of invalidity of the NS Patents. Sarepta is seeking an award of relief in its defenses to NS' allegations, a judgment of breach of contract, a determination of invalidity of the NS Patents, a judgment of infringement and willful infringement of the UWA Patents, legal fees and costs, an unspecified amount of monetary relief (treble damages) attributable to NS' alleged infringement, and such other relief as the court deems just and proper. UWA has since been joined as a Plaintiff in Sarepta's counterclaims against NS. The Court entered a scheduling order with a trial scheduled to commence on May 13, 2024.

On or about June 5, 2023, Sarepta initiated a patent infringement lawsuit against Nippon Shinyaku in Japan, alleging that NS's production, sales and offers to sell Viltepso infringe Sarepta's Japanese Patent No. 6406782. NS filed its preliminary answer on July 13, 2023. A hearing occurred on July 20, 2023 during which the Court set an initial case schedule.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The purpose of Management's Discussion and Analysis of Financial Condition and Results of Operations is to provide an understanding of the financial condition, changes in financial condition and results of operations of Sarepta Therapeutics, Inc. This section should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and the section contained in our Annual Report on Form 10-K for the year ended December 31, 2022 under the caption "Part II-Item 7 — Management's Discussion and Analysis of Financial Condition and Results of Operations". This Quarterly Report on Form 10-Q contains certain forward-looking statements, which are often identified by words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may," "estimate," "could," "continue," "ongoing," "predict," "potential," "likely," "seek" and other similar expressions, as well as variations or negatives of these words. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to:

- *our belief that our proprietary technology platforms and collaborations can be used to develop potential therapeutic candidates to treat a broad range of diseases;*
- *our expectation that our partnerships with manufacturers will support our clinical and commercial manufacturing capacity for our Duchenne muscular dystrophy gene therapy programs and Limb-girdle muscular dystrophy programs, while also acting as a manufacturing platform for potential future gene therapy programs, and our belief that our current network of manufacturing partners is able to fulfill the requirements of our commercial plan;*
- *the possible impact of regulations and regulatory decisions by the Food and Drug Administration (the "FDA") and other regulatory agencies on our business as well as the development of our product candidates and our financial and contractual obligations;*
- *estimated timelines and milestones for the remainder of 2023 and beyond, including engaging with the FDA to discuss our next steps for our SRP-9003 program in 2023, top-line results from Study 9001-301 in the fourth quarter of 2023, top-line results from Part B of Study 5051-201 by the end of 2023 and the potential to expand the label of ELEVIDYS;*
- *our engagement with regulatory authorities outside of the U.S. including the EMA;*
- *our plan to continue building out our network for commercial distribution in jurisdictions in which our products are approved;*
- *our plan to expand our pipeline through internal research and development and through strategic transactions;*
- *the timely completion and satisfactory outcome of our post-marketing requirements and commitments, including verification of a clinical benefit for our products in confirmatory trials;*
- *our ability to further secure long-term supply of our commercial products and our product candidates to satisfy our planned commercial, early access programs ("EAP") and clinical needs;*
- *the possible impact of any competing products on the commercial success of our products and our product candidates and our ability to compete against such products;*
- *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; our plans and ability to file and progress to issue additional patent applications to enhance and protect our new and existing technologies and programs;*
- *the potential impact of the ongoing COVID-19 pandemic on our business, including our commercial sales, ongoing and planned clinical trials, manufacturing and operations;*
- *our estimates regarding our cash runway and statements about our future capital needs;*
- *our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;*

- *our expectation regarding the impact of environmental laws and regulations on our business; and*
- *our beliefs and expectations regarding milestone, royalty or other payments that could be due to third parties under existing agreements.*

We undertake no obligation to update any of the forward-looking statements contained in this Quarterly Report on Form 10-Q after the date of this report, except as required by law or the rules and regulations of the U.S. Securities and Exchange Commission (the "SEC"). *We caution readers not to place undue reliance on forward-looking statements. Our actual results could differ materially from those discussed in this Quarterly Report on Form 10-Q. The forward-looking statements contained in this Quarterly Report on Form 10-Q, 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 Quarterly Report on Form 10-Q.*

Overview

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

We commercialized four products, all of which were granted accelerated approval by the FDA:

- EXONDYS 51 (eteplirsen) Injection ("EXONDYS 51"), approved by the FDA on September 19, 2016, is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 51 skipping. EXONDYS 51 uses our phosphorodiamidate morpholino oligomer ("PMO") chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene.
- VYONDYS 53 (golodirsen) Injection ("VYONDYS 53"), approved by the FDA on December 12, 2019, is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 53 skipping. VYONDYS 53 uses our PMO chemistry and exon-skipping technology to skip exon 53 of the dystrophin gene.
- AMONDYS 45 (casimersen) Injection ("AMONDYS 45"), approved by the FDA on February 25, 2021, is indicated for the treatment of Duchenne in patients who have a confirmed mutation of the dystrophin gene that is amenable to exon 45 skipping. AMONDYS 45 uses our PMO chemistry and exon-skipping technology to skip exon 45 of the dystrophin gene.
- ELEVIDYS (delandistrogene moxeparvovec-rokl), approved by the FDA on June 22, 2023, is an adeno-associated virus based gene therapy for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne with a confirmed mutation in the Duchenne gene. ELEVIDYS is contraindicated in patients with any deletion in exon 8 and/or exon 9 in the Duchenne gene.

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

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

- *SRP-5051* uses our next-generation chemistry platform, cell-penetrating peptide-conjugated PPMO, and our exon-skipping technology to skip exon 51 of the dystrophin gene. SRP-5051, a peptide conjugated PMO, is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein. In the fourth quarter of 2017, we commenced a first-in-human, single ascending dose, study for the treatment of Duchenne in patients who are amenable to exon 51 skipping. In 2019, we commenced Study 5051-201. In December 2020, we announced an interim analysis on clinical results from the 10 mg/kg and 20 mg/kg dose cohorts of Part A of Study 5051-201. In May 2021, we announced results from the 30 mg/kg cohort of Part A of Study 5051-201. We initiated Part B of Study 5051-201 in the fourth quarter of 2021. In July 2022, the FDA placed Study 5051-201 on clinical hold following a serious adverse event of hypomagnesemia. The clinical hold was lifted in August 2022. We completed enrollment of Part B of Study 5051-201 in the first quarter of 2023 and anticipate top-line results from Part B in the second half of 2023. If successful, we anticipate Part B to be our potentially pivotal trial.

- *SRP-9003* (LGMD, gene therapy program). We are developing gene therapy programs for various forms of LGMDs. The most advanced of our LGMD product candidates, SRP-9003, is designed to transfer a gene that codes for and restores beta-sarcoglycan protein with the goal of restoring the dystrophin associated protein complex. It utilizes the AAVrh.74 vector system, the same vector used in our SRP-9001 gene therapy program. A Phase 1/2a trial of SRP-9003 was commenced in the fourth quarter of 2018. In February 2019, we announced positive two-month biopsy data from the first three-patient low-dose cohort dosed in the SRP-9003 trial, and in October 2019, we announced positive nine-month functional data from these three patients. We have dosed one additional cohort of three patients at a higher dose per the study protocol. In June 2020, we announced safety and expression results from three clinical trial participants in the high-dose cohort measured at 60 days, and one-year functional data from three clinical trial participants in the low-dose cohort. In September 2020, we announced six-month functional data from three clinical trial participants in the high-dose cohort, and eighteen-month functional data from three clinical trial participants in the low-dose cohort. In March 2021, we announced 24-month functional and expression data from the three clinical trial participants in the low-dose cohort and twelve-month functional data from the three clinical trial participants in the high-dose cohort. In March 2022, we announced 36-month functional data from three clinical trial participants in the low-dose cohort and 24-month functional data from two clinical trial participants in the high-dose cohort. We plan to meet with the FDA in 2023 to discuss our potentially pivotal trial.

In addition, we are also seeking to expand the label of ELEVIDYS and initiated our Study 301 in October 2021. We anticipate top-line results in the fourth quarter of 2023.

Our pipeline includes more than 40 programs in various stages of pre-clinical and clinical development, reflecting our multifaceted approach and expertise in precision genetic medicine to make a profound difference in the lives of patients suffering from rare diseases.

Manufacturing, Supply and Distribution

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

For our commercial Duchenne PMO program, we have worked with our existing CMOs to increase production capacity from mid-scale to large-scale. While there 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 commercial products, based on our diligence to date, we believe our current network of CMOs is able to fulfill these requirements, and is capable of expanding capacity as needed. Additionally, we have, and will continue to evaluate further relationships with additional suppliers to increase overall capacity as well as further reduce risks associated with reliance on a limited number of suppliers for manufacturing.

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

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

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

Cash, Cash Equivalents, Restricted Cash and Investments

As of June 30, 2023, we had approximately $1,879.7 million of cash, cash equivalents, restricted cash and investments, consisting of $851.9 million of cash and cash equivalents, $1,008.8 million of short-term investments and $19.0 million of long-term restricted cash. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next 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 reimbursement programs and the complex regulatory environment in which we operate.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations is based upon our unaudited condensed consolidated financial statements included elsewhere in this report. The preparation of our unaudited condensed 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 unaudited condensed 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 unaudited condensed consolidated financial statements:

- inventory; and
- income tax.

Aside from the removal of stock-based compensation as a critical accounting policy as of January 1, 2023, there have been no changes to our critical accounting policies and significant estimates as detailed in our Annual Report on Form 10-K for the year ended December 31, 2022.

Results of Operations for the Three and Six Months Ended June 30, 2023 and 2022

The following tables set forth selected unaudited condensed consolidated statements of operations data for each of the periods indicated:

	For the Three Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Products, net	$ 238,988	$ 211,237	$ 27,751	13 %
Collaboration	22,250	22,250	—	(—)%
Total revenues	261,238	233,487	27,751	12 %
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	34,124	37,795	(3,671)	(10)%
Research and development	241,890	252,329	(10,439)	(4)%
Selling, general and administrative	118,564	154,316	(35,752)	(23)%
Amortization of in-licensed rights	179	179	—	(—)%
Total cost and expenses	394,757	444,619	(49,862)	(11)%
Operating loss	(133,519)	(211,132)	77,613	37 %
Other income (loss), net:				
Gain from sale of Priority Review Voucher	102,000	—	102,000	NM*
Other income (expense), net	16,934	(16,961)	33,895	200 %
Total other income (loss), net	118,934	(16,961)	135,895	NM*
Loss before income tax expense	(14,585)	(228,093)	213,508	94 %
Income tax expense	9,355	3,388	5,967	176 %
Net loss	$ (23,940)	$ (231,481)	$ 207,541	90 %
Net loss per share - basic and diluted	$ (0.27)	$ (2.65)	$ 2.38	(90)%

	For the Six Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Products, net	$ 470,483	$ 400,062	$ 70,421	18 %
Collaboration	44,255	44,255	—	(—)%
Total revenues	514,738	444,317	70,421	16 %
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	69,141	69,238	(97)	(—)%
Research and development	487,569	446,579	40,990	9 %
Selling, general and administrative	229,278	226,156	3,122	1 %
Amortization of in-licensed rights	357	357	—	(—)%
Total cost and expenses	786,345	742,330	44,015	6 %
Operating loss	(271,607)	(298,013)	26,406	9 %
Other loss, net				
Gain from sale of Priority Review Voucher	102,000	—	102,000	NM*
Loss on debt extinguishment	(387,329)	—	(387,329)	NM*
Other income (expense), net	29,641	(34,226)	63,867	187 %
Total other loss, net	(255,688)	(34,226)	(221,462)	NM*
Loss before income tax expense	(527,295)	(332,239)	(195,056)	(59)%
Income tax expense	13,400	4,267	9,133	214 %
Net loss	$ (540,695)	$ (336,506)	$ (204,189)	(61)%
Net loss per share - basic and diluted	$ (6.11)	$ (3.85)	$ (2.26)	(59)%

* NM: not meaningful

Revenues

Revenues from product sales are recorded at the time of sale at the net sales price (transaction price), which includes estimates of variable consideration for which reserves are established and which result from Medicaid rebates, governmental chargebacks including Public Health Services chargebacks, prompt pay discounts, co-pay assistance and distribution fees. These reserves are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable (if no payments are required of us) or a current liability (if a payment is required of us). Our estimates take into consideration current contractual and statutory requirements. Actual amounts of consideration ultimately received or paid may differ from our estimates.

The following tables summarize the components of our net product revenues, by product, for each of the periods indicated:

	For the Three Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands)		$	%
EXONDYS 51	$ 134,688	$ 126,377	$ 8,311	7%
AMONDYS 45	71,653	54,676	16,977	31%
VYONDYS 53	32,647	30,184	2,463	8%
Products, net	$ 238,988	$ 211,237	$ 27,751	13%

	For the Six Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands)		$	%
EXONDYS 51	$ 267,259	$ 243,510	$ 23,749	10%
AMONDYS 45	137,565	98,290	39,275	40%
VYONDYS 53	65,659	58,262	7,397	13%
Products, net	$ 470,483	$ 400,062	$ 70,421	18%

Net product revenues for our products for the three and six months ended June 30, 2023 increased by $27.8 million and $70.4 million, respectively, compared with the three and six months ended June 30, 2022. The increase primarily reflects increasing demand for EXONDYS 51, AMONDYS 45 and VYONDYS 53 (collectively, “PMO Products”). There were no product sales related to ELEVIDYS during the three or six months ended June 30, 2023.

Collaboration revenue relates to our collaboration arrangement with F. Hoffman-La Roche Ltd. (“Roche”). For both the three and six months ended June 30, 2023 and 2022, we recognized $22.3 million and $44.3 million of collaboration revenue, respectively. For more information, please read *Note 3, License and Collaboration Agreements* to the financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2022.

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

Our cost of sales (excluding amortization of in-licensed rights) consists of royalty payments primarily to BioMarin Pharmaceuticals, Inc. (“BioMarin”) and the University of Western Australia (“UWA”), inventory costs that relate to sales of our products and the related overhead costs. Prior to receiving regulatory approval for EXONDYS 51, VYONDYS 53, AMONDYS 45 and ELEVIDYS by the FDA in September 2016, December 2019, February 2021 and June 2023, respectively, we expensed such manufacturing and material costs as research and development expenses. For AMONDYS 45 sold in the three and six months ended June 30, 2022, 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 the product. If PMO product related costs had not previously been expensed as research and development expenses prior to receiving FDA approval, the incremental inventory costs related to our PMO Products sold would have been approximately $4.2 million and $6.7 million for the three and six months ended June 30, 2022, respectively.

The following tables summarize the components of cost of sales for our PMO Products (excluding amortization of in-licensed rights) for each of the periods indicated:

	For the Three Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands)		$	%
Inventory costs related to products sold	$ 25,541	$ 23,050	$ 2,491	11%
Royalty payments	8,583	14,745	(6,162)	(42)%
Total cost of sales (excluding amortization of in-licensed rights)	$ 34,124	$ 37,795	$ (3,671)	(10)%

	For the Six Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands)		$	%
Inventory costs related to products sold	$ 52,175	$ 41,513	$ 10,662	26 %
Royalty payments	16,966	27,725	(10,759)	(39)%
Total cost of sales (excluding amortization of in-licensed rights)	$ 69,141	$ 69,238	$ (97)	(—)%

The cost of sales (excluding amortization of in-licensed rights) for the three months ended June 30, 2023 decreased by $3.7 million, or 10%, compared with the same period in 2022. The change primarily reflects a decrease in royalty payments during the three months ended June 30, 2023 due to changes in the BioMarin royalty terms and a decrease in write-offs of certain batches of our products not meeting our quality specifications for the three months ended June 30, 2023, as compared to the same period of 2022, partially offset by an increasing demand for our PMO Products.

The cost of sales (excluding amortization of in-licensed rights) for the six months ended June 30, 2023 slightly decreased by $0.1 million, compared with the same period in 2022. The change primarily reflects a decrease in royalty payments during the six months ended June 30, 2023 due to changes in the BioMarin royalty terms, offset by an increasing demand for our PMO Products and an increase in write-offs of certain batches of our products not meeting our quality specifications for the six months ended June 30, 2023, as compared to the same period of 2022.

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

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

The following tables summarize our research and development expenses by project for each of the periods indicated:

	For the Three Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands)		$	%
SRP-9001	$ 88,481	$ 130,583	$ (42,102)	(32)%
Eteplirsen (exon 51)	29,358	14,693	14,665	100 %
Other gene therapies	20,391	19,382	1,009	5 %
PPMO platform	17,445	11,156	6,289	56 %
Up-front, milestone and other expenses	7,600	11,300	(3,700)	(33)%
Casimersen (exon 45)	5,367	9,574	(4,207)	(44)%
Golodirsen (exon 53)	2,890	3,365	(475)	(14)%
Collaboration cost-sharing	2,451	1,474	977	66 %
Other projects	4,247	6,150	(1,903)	(31)%
Internal research and development expenses	91,532	70,947	20,585	29 %
Roche collaboration reimbursement	(27,872)	(26,295)	(1,577)	6 %
Total research and development expenses	$ 241,890	$ 252,329	$ (10,439)	(4)%

	For the Six Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands)		$	%
SRP-9001	$ 193,100	$ 222,144	$ (29,044)	(13)%
Eteplirsen (exon 51)	51,972	23,749	28,223	119%
Other gene therapies	40,292	35,259	5,033	14%
PPMO platform	32,541	26,122	6,419	25%
Casimersen (exon 45)	13,030	17,413	(4,383)	(25)%
Golodirsen (exon 53)	8,405	8,171	234	3%
Up-front, milestone and other expenses	8,097	11,300	(3,203)	(28)%
Collaboration cost-sharing	3,600	2,558	1,042	41%
Other projects	8,391	7,749	642	8%
Internal research and development expenses	176,136	136,022	40,114	29%
Roche collaboration reimbursement	(47,995)	(43,908)	(4,087)	9%
Total research and development expenses	$ 487,569	$ 446,579	$ 40,990	9%

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

	For the Three Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands)		$	%
Manufacturing expenses	$ 98,999	$ 150,279	$ (51,280)	(34)%
Compensation and other personnel expenses	45,132	34,450	10,682	31%
Clinical trial expenses	42,508	28,817	13,691	48%
Facility- and technology-related expenses	25,224	20,258	4,966	25%
Stock-based compensation	21,577	14,467	7,110	49%
Professional services	8,062	5,332	2,730	51%
Up-front, milestone and other expenses	7,600	11,300	(3,700)	(33)%
Pre-clinical expenses	2,726	898	1,828	204%
Collaboration cost-sharing	2,451	1,474	977	66%
Research and other	15,483	11,349	4,134	36%
Roche collaboration reimbursement	(27,872)	(26,295)	(1,577)	6%
Total research and development expenses	$ 241,890	$ 252,329	$ (10,439)	(4)%

Research and development expenses for the three months ended June 30, 2023 decreased by $10.4 million, or 4%, compared with the three months ended June 30, 2022. The decrease was primarily driven by the following:

- $51.3 million decrease in manufacturing expenses primarily due to the $53.0 million shortfall payment accrual related to the gene therapy manufacturing and supply agreement with Thermo and the $17.1 million termination charge related to the manufacturing and supply agreement with Henogen SA, both of which occurred in the three months ended June 30, 2022, with no similar activity in the same period of 2023, partially offset by a continuing ramp-up of SRP-9001 manufacturing prior to the ELEVIDYS approval in June 2023;
- $10.7 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $13.7 million increase in clinical trial expenses primarily due to an increased patient enrollment and site activation for our MOMENTUM, ENVISION and MIS51ON programs;
- $5.0 million increase in facility- and technology-related expenses primarily due to our continuing expansion efforts;
- $7.1 million increase in stock-based compensation expense primarily due to the achievement of one performance condition related to certain restricted stock units with performance conditions (“PSUs”) during the three months ended June 30, 2023, as well as changes in headcount and the value of stock awards;
- $2.7 million increase in professional service expenses primarily due to an increase in reliance on third-party research and development contractors for the launch of ELEVIDYS;
- $3.7 million decrease in up-front, milestone and other expenses primarily due to $2.8 million of up-front payments as a result of the execution of certain research and license agreements, $4.0 million of expense incurred as a result of milestone achievements in certain research and license agreements and $4.5 million of option and termination expenses in the three months ended June 30, 2022, partially offset by $7.5 million of up-front payments as a result of the execution of certain research and license agreements in the same period of 2023;

- $1.8 million increase in pre-clinical expenses primarily due to an increase in toxicology study activity across multiple gene therapy and RNA platforms;
- $4.1 million increase in research and other expenses primarily driven by an increase in sponsored research with academic institutions during the three months ended June 30, 2023 and an increase in lab-related expenses; and
- $1.6 million increase in the offset to expense associated with a collaboration reimbursement from Roche due to continuing development of our SRP-9001 gene therapy programs.

	For the Six Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands)		$	%
Manufacturing expenses	$ 210,920	$ 246,568	$ (35,648)	(14)%
Compensation and other personnel expenses	91,318	66,646	24,672	37%
Clinical trial expenses	83,400	59,255	24,145	41%
Facility- and technology-related expenses	48,569	40,772	7,797	19%
Stock-based compensation	37,990	27,535	10,455	38%
Professional services	14,573	9,172	5,401	59%
Up-front, milestone and other expenses	8,097	11,300	(3,203)	(28)%
Pre-clinical expenses	6,189	5,866	323	6%
Collaboration cost-sharing	3,600	2,558	1,042	41%
Research and other	30,908	20,815	10,093	48%
Roche collaboration reimbursement	(47,995)	(43,908)	(4,087)	9%
Total research and development expenses	$ 487,569	$ 446,579	$ 40,990	9%

Research and development expenses for the six months ended June 30, 2023 increased by $41.0 million, or 9%, compared with the six months ended June 30, 2022. The increase was primarily driven by the following:

- $35.6 million decrease in manufacturing expenses primarily due to the $53.0 million shortfall payment accrual related to the gene therapy manufacturing and supply agreement with Thermo and the $17.1 million termination charge related to the manufacturing and supply agreement with Henogen SA, both of which occurred in the three months ended June 30, 2022, with no similar activity in the same period of 2023, partially offset by a continuing ramp-up of SRP-9001 manufacturing prior to the ELEVIDYS approval in June 2023;
- $24.7 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $24.1 million increase in clinical trial expenses primarily due to an increased patient enrollment and site activation for our MOMENTUM and MIS51ON programs;
- $7.8 million increase in facility- and technology-related expenses primarily due to our continuing expansion efforts;
- $10.5 million increase in stock-based compensation expense primarily due to changes in headcount and the value of stock awards, as well as the achievement of one performance condition related to certain PSUs during the three months ended June 30, 2023;
- $5.4 million increase in professional service expenses primarily due to an increase in reliance on third-party research and development contractors for the launch of ELEVIDYS;
- $3.2 million decrease in up-front, milestone and other expenses primarily due to $2.8 million of up-front payments as a result of the execution of certain research and license agreements, $4.0 million of expense incurred as a result of milestone achievements in certain research and license agreements and $4.5 million of option and termination expenses in the six months ended June 30, 2022, partially offset by $7.8 million of up-front payments as a result of the execution of certain research and license agreements in the same period of 2023;
- $10.1 million increase in research and other expenses primarily driven by an increase in sponsored research with academic institutions during the six months ended June 30, 2023 and an increase in lab-related expenses; and
- $4.1 million increase in the offset to expense associated with a collaboration reimbursement from Roche due to continuing development of our SRP-9001 gene therapy programs.

Selling, general and administrative expenses

Selling, general and administrative expenses consist of salaries, benefits, stock-based compensation and related costs for personnel in our executive, finance, legal, information technology, business development, human resources, commercial and other

general and administrative functions. Other general and administrative expenses include an allocation of our facility- and technology-related costs and professional fees for legal, consulting and accounting services.

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

	For the Three Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands)		$	%
Compensation and other personnel expenses	$ 38,329	$ 28,622	$ 9,707	34 %
Professional services	37,797	25,772	12,025	47 %
Stock-based compensation	25,800	88,425	(62,625)	(71)%
Facility- and technology-related expenses	10,416	8,253	2,163	26 %
Other	6,550	3,370	3,180	94 %
Roche collaboration reimbursement	(328)	(126)	(202)	160 %
Total selling, general and administrative expenses	$ 118,564	$ 154,316	$ (35,752)	(23)%

Selling, general and administrative expenses for the three months ended June 30, 2023 decreased by $35.8 million, or 23%, compared with the three months ended June 30, 2022. This decrease was primarily driven by the following:

- $9.7 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $12.0 million increase in professional service expenses primarily due to an increase in reliance on third-party selling, general and administrative contractors for the launch of ELEVIDYS;
- $62.6 million decrease in stock-based compensation expense primarily related to the Chief Executive Officer grant modification agreement executed in 2022, partially offset by changes in headcount and the value of stock awards and the achievement of one performance condition related to certain PSUs during the three months ended June 30, 2023;
- $2.2 million increase in facility- and technology-related expenses primarily due to our continuing expansion efforts; and
- $3.2 million increase in other expenses primarily due to changes in charitable contribution activity.

	For the Six Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands)		$	%
Compensation and other personnel expenses	$ 76,796	$ 56,075	$ 20,721	37 %
Professional services	72,888	43,066	29,822	69 %
Stock-based compensation	50,637	104,555	(53,918)	(52)%
Facility- and technology-related expenses	20,266	16,406	3,860	24 %
Other	9,176	6,289	2,887	46 %
Roche collaboration reimbursement	(485)	(235)	(250)	106 %
Total selling, general and administrative expenses	$ 229,278	$ 226,156	$ 3,122	1 %

Selling, general and administrative expenses for the six months ended June 30, 2023 increased by $3.1 million, or 1%, compared with the six months ended June 30, 2022. This increase was primarily driven by the following:

- $20.7 million increase in compensation and other personnel expenses primarily due to changes in headcount;
- $29.8 million increase in professional service expenses primarily due to an increase in reliance on third-party selling, general and administrative contractors for the launch of ELEVIDYS;
- $53.9 million decrease in stock-based compensation expense primarily related to the Chief Executive Officer grant modification agreement executed in 2022, partially offset by changes in headcount and the value of stock awards and the achievement of one performance condition related to certain PSUs during the six months ended June 30, 2023;
- $3.9 million increase in facility- and technology-related expenses primarily due to our continuing expansion efforts; and
- $2.9 million increase in other expenses primarily due to changes in charitable contribution activity.

Amortization of in-licensed rights

Amortization of in-licensed rights relates to the agreements we entered into with UWA, Nationwide Children's Hospital ("Nationwide"), BioMarin, and Parent Project Muscular Dystrophy in April 2013, December 2016, July 2017 and May 2018, respectively. Each in-licensed right is being amortized on a straight-line basis over the remaining life of the relevant patent from the date the related fee was incurred, either the regulatory approval of or the first commercial sale of the applicable product. For both the three and six months ended June 30, 2023 and 2022, we recorded amortization of in-licensed rights of approximately $0.2 million and $0.4 million, respectively.

Gain from sale of Priority Review Voucher

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

Loss on debt extinguishment

On November 14, 2017, we issued $570.0 million aggregate principal amount of senior convertible notes due on November 15, 2024 (the "2024 Notes"). On March 2, 2023, we entered into separate, privately negotiated exchange agreements with certain holders of the outstanding 2024 Notes (the "Exchange Agreements"). The Exchange Agreements resulted in an exchange of $313.5 million in aggregate principal value of the 2024 Notes for shares of our common stock (the "2024 Notes Exchange"). In connection with the 2024 Notes Exchange, we issued approximately 4.5 million shares of our common stock representing an agreed upon contractual exchange rate pursuant to the terms of each Exchange Agreement. The exchange was not pursuant to the conversion privileges included in the terms of the debt at issuance and therefore was accounted for as a debt extinguishment. We accounted for the debt extinguishment by recognizing the difference between the fair value of the shares of common stock transferred on the exchange date and the net carrying amount of the extinguished debt as a loss on debt extinguishment. The loss incurred on the extinguishment for the six months ended June 30, 2023 was $387.3 million, inclusive of $6.9 million in third-party debt conversion costs.

Other income (expense), net

Other income (expense), net primarily consists of interest expense on our debt facilities, interest income on our cash, cash equivalents and investments, amortization of investment premium or accretion of investment discount and unrealized gain or loss from our investment in our strategic investments. Interest expense primarily includes interest accrued on our convertible notes. Our cash equivalents and investments consist of money market funds, corporate bonds, commercial paper, government and government agency debt securities and certificates of deposit.

For the three and six months ended June 30, 2023, other income, net, was $16.9 million and $29.6 million, respectively. For the three and six months ended June 30, 2022, other expense, net was $17.0 million and $34.2 million, respectively. The changes are primarily due to increases in accretion of investment discount, net and increases in interest income due to the investment mix of our investment portfolio, as well as a reductions of interest expense incurred as a result of the repayment of our December 2019 Term Loan in 2022.

Income tax expense

Income tax expense for the three and six months ended June 30, 2023 was approximately $9.4 million and $13.4 million, respectively. Income tax expense for the three and six months ended June 30, 2022 was approximately $3.4 million and $4.3 million, respectively. Income tax expense for the three and six months ended June 30, 2023 relates to state, foreign and federal income taxes, while income tax expense for the three and six months ended June 30, 2022 relates to state and foreign income taxes.

Liquidity and Capital Resources

Refer to *Note 11, Indebtedness* and *Note 14, Leases*, for additional discussion surrounding material changes to our obligations under outstanding indebtedness and lease arrangements, respectively.

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

	As of June 30, 2023	As of December 31, 2022	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 851,929	$ 966,777	$ (114,848)	(12)%
Short-term investments	1,008,786	1,022,597	(13,811)	(1)%
Restricted cash and investments	19,024	19,024	—	(—)%
Total cash, cash equivalents and investments	$ 1,879,739	$ 2,008,398	$ (128,659)	(6)%
Borrowings:				
Convertible debt	$ 1,235,517	$ 1,544,292	$ (308,775)	(20)%
Total borrowings	$ 1,235,517	$ 1,544,292	$ (308,775)	(20)%
Working capital				
Current assets	$ 2,472,614	$ 2,557,861	$ (85,247)	(3)%
Current liabilities	498,654	619,604	(120,950)	(20)%
Total working capital	$ 1,973,960	$ 1,938,257	$ 35,703	2%

For the periods ended June 30, 2023 and December 31, 2022, our principal sources of liquidity were primarily derived from sales of our products, net proceeds from sale of the ELEVIDYS PRV, net proceeds from our offering of 1.25% convertible senior notes due on September 15, 2027 (“2027 Notes”) and our collaboration arrangement with Roche. Our principal uses of cash are research and development expenses, selling, general and administrative expenses, investments, capital expenditures, business development transactions and other working capital requirements. The changes in our working capital primarily reflect use of cash in operating activities. While our contractual obligations, commitments and debt service requirements over the next several years are significant, we intend to continue to fund our short-term financing needs and working capital requirements from cash flows of operating activities as well as cash on hand, and such sources are anticipated to be adequate to fund working capital requirements for at least twelve months from the date these unaudited condensed consolidated financial statements were issued.

Beyond June 30, 2024, our cash requirements will depend extensively on our ability to advance our research, development and commercialization of product candidates. We may seek additional financings primarily from, but not limited to, the sale and issuance of equity and debt securities, the licensing or sale of our technologies, and entering into additional government contracts and/or funded research and development agreements. Our future expenditures and long-term capital requirements may be substantial and will depend on many factors, including but not limited to the following:

- our ability to continue to generate revenues from sales of commercial products and potential future products;
- the timing and costs associated with our expansion efforts;
- the timing and costs of building out our manufacturing capabilities;
- the timing of payments related to our future inventory commitments and manufacturing obligations;
- the timing and costs associated with our existing lease obligations and new obligations expected to be entered into during the following year;
- the timing and costs associated with our clinical trials and pre-clinical trials;
- the attainment of milestones and our obligations to make milestone payments to Myonexus's selling shareholders, BioMarin, Nationwide, UWA and other institutions;
- obligations to holders of our convertible notes; and
- the costs of filing, prosecuting, defending and enforcing patent claims and our other intellectual property rights.

We cannot provide assurances that financing will be available when and as needed or that, if available, the financings will be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we require, this would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

We have entered into long-term contractual arrangements from time to time for our facilities, the provision of goods and services, and issuance of debt securities, among others. As of June 30, 2023, total obligations under debt, lease, and manufacturing

arrangements were $1.3 billion, $363.3 million, and $1.3 billion, respectively with $15.9 million, $20.0 million and $824.0 million due in less than one year, and approximately $1,307.0 million, $343.3 million and $457.8 million due in greater than one year. Interest payments are included within the future debt obligations stated in the previous sentence. Lease obligations only include real estate leases that had commenced prior to June 30, 2023. The leases embedded in a certain supply agreement are included in manufacturing obligations. Additional information regarding our obligations under debt and manufacturing arrangements is provided in *Note 11, Indebtedness* and *Note 16, Commitments and Contingencies*, respectively, to the unaudited condensed consolidated financial statements contained in Item 1.

For products and product candidates that are currently in various research and development stages, we may be obligated to make up to $3.2 billion of future development, regulatory, commercial and up-front royalty and sales milestone payments associated with our license and collaboration agreements. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory or commercial milestones. Because the achievement of these milestones is not probable, and payment is not required as of June 30, 2023, such contingencies have not been recorded in our unaudited condensed consolidated financial statements. Amounts related to contingent milestone payments are not yet considered contractual obligations as they are contingent on the successful achievement of certain development, regulatory approval and commercial milestones.

Cash Flows

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

	For the Six Months Ended June 30,			
	2023	2022	Change	Change
	(in thousands)		$	%
Cash (used in) provided by				
Operating activities	$ (331,630)	$ (167,990)	$ (163,640)	97%
Investing activities	109,126	(1,075,798)	1,184,924	(110)%
Financing activities	107,656	5,329	102,327	NM*
Decrease in cash and cash equivalents	$ (114,848)	$ (1,238,459)	$ 1,123,611	(91)%

* NM: not meaningful

Operating Activities

Cash used in operating activities, which consists of our net loss adjusted for non-cash items and changes in net operating assets and liabilities, totaled $331.6 million and $168.0 million for the six months ended June 30, 2023 and 2022, respectively. Cash used in operating activities for the six months ended June 30, 2023 was primarily driven by the net loss of $540.7 million, adjusted for the following:

- $387.3 million in loss on debt extinguishment related to the exchange of 2024 Notes;
- $88.6 million in stock-based compensation expense;
- $22.1 million in depreciation and amortization expense; and
- $9.8 million in other non-cash items.

These amounts were partially offset by the gain of $102.0 million recorded from the sale of the ELEVIDYS PRV and $20.2 million in accretion of investment discount, net.

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

- $93.7 million decrease in accounts payable, accrued expenses, lease liabilities and other liabilities, primarily due to the $54.0 million shortfall payment to Thermo made during the six months ended June 30, 2023, payments to Catalent for raw materials and the overall timing and invoicing of payments;
- $44.3 million decrease in deferred revenue related to the collaboration with Roche;
- $27.0 million increase in inventory due to the addition of capitalized inventory after the approval of ELEVIDYS in June 2023, partially offset by a decrease in inventory for our PMO Products; and
- $22.2 million increase in accounts receivable due to an increase in the demand of our PMO Products.

These amounts were partially offset by $11.0 million decrease in other assets primarily due to a decrease in the collaboration receivable related to Roche and timing of manufacturing prepaids.

Cash used in operating activities for the six months ended June 30, 2022 was primarily driven by the net loss of $336.5 million, adjusted for the following:

- $132.1 million in stock-based compensation expense;
- $20.6 million in depreciation and amortization expense; and
- $13.9 million in other non-cash items.

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

- $50.9 million increase in accounts receivable due to an increase in the demand of our PMO Products;
- $44.3 million decrease in deferred revenue related to the collaboration with Roche; and
- $15.4 million increase in inventory due to our continuing build-up of inventory corresponding to the increase in demand for our PMO Products.

These amounts were partially offset by a $30.9 million decrease in other assets primarily due to lower consumption of manufacturing-related deposits during the period and an increase of $81.5 million in accounts payable, accrued expenses, lease liabilities and other liabilities, primarily due to an accrual for the estimated shortfall payment to Thermo and the timing and invoicing of payments.

Investing Activities

Cash provided by investing activities was $109.1 million for the six months ended June 30, 2023, compared to $1,075.8 million of cash used in the six months ended June 30, 2022. Cash provided by investing activities for the six months ended June 30, 2023 primarily consisted of $102.0 million of net proceeds related to the sale of the ELEVIDYS PRV and $864.5 million from the maturity and sale of available-for-sale securities, partially offset by $829.8 million of purchases of available-for-sale securities and $27.4 million of purchases of property and equipment.

Cash used in investing activities for the six months ended June 30, 2022 primarily consisted of $1,137.6 million of purchases of available-for-sale securities and $14.6 million of purchases of property and equipment, partially offset by proceeds of $77.2 million from the maturity of available-for-sale securities.

Financing Activities

Cash provided by financing activities was $107.7 million and $5.3 million for the six months ended June 30, 2023 and 2022, respectively. Cash provided by financing activities for the six months ended June 30, 2023 consisted of $80.6 million in partial settlement of capped call share options for the 2024 Notes and $33.9 million in proceeds from exercise of options and purchase of stock under our Employee Stock Purchase Program, partially offset by $6.9 million in third-party debt conversion costs related to the 2024 Notes Exchange.

Cash provided by financing activities for the six months ended June 30, 2022 consisted of $5.3 million in proceeds from exercise of options and purchase of stock under our Employee Stock Purchase Program.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our current investment policy is to maintain a diversified investment portfolio consisting of money market investments, commercial paper, certificates of deposit, government and government agency bonds and high-grade corporate bonds with maturities of 36 months or less. Our cash is deposited in and invested through highly rated financial institutions. As of June 30, 2023, we had approximately $1,879.7 million of cash, cash equivalents, restricted cash and investments, comprised of $851.9 million of cash and cash equivalents, $1,008.8 million of short-term investments and $19.0 million long-term restricted cash. 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. As of June 30, 2023, we estimate that such hypothetical 10 basis point movement would result in a hypothetical loss in fair value of approximately $0.3 million to our interest rate sensitive instruments.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

We carried out an evaluation as of the end of the period covered by this Quarterly Report on Form 10-Q for the period ended June 30, 2023, under the supervision and with the participation of our management, including our principal executive officer and our principal financial officer, of the effectiveness of our disclosure controls and procedures pursuant to paragraph (b) of Rules 13a-15 and 15d-15 under the Securities Exchange Act of 1934, as amended (the "Exchange Act"). The purpose of this evaluation was to determine whether as of the evaluation date our disclosure controls and procedures were effective to provide reasonable assurance that the information we are required to disclose in our filings with the SEC under the Exchange Act (i) is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms and (ii) is accumulated and communicated to our management, including our principal executive officer and our principal financial officer, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of June 30, 2023, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarterly period ended June 30, 2023, there were no changes in our internal controls over financial reporting that have materially affected or are reasonably likely to materially affect our internal control over financial reporting.

PART II OTHER INFORMATION

Item 1. Legal Proceedings

For material legal proceedings, please read *Note 16, Commitments and Contingencies* to our unaudited condensed consolidated financial statements included in this report.

Item 1A. Risk Factors.

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

Risks Related to Our Business

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

The FDA granted accelerated approval for EXONDYS 51, VYONDYS 53, AMONDYS 45 and ELEVIDYS, respectively, as therapeutic treatments for Duchenne in patients who have a confirmed mutation in the dystrophin gene that is amenable to exon 51, exon 53, exon 45 skipping, and ambulatory pediatric patients aged 4 through 5 years with Duchenne with a confirmed mutation in the Duchenne gene, respectively. EXONDYS 51 has been approved for marketing in the U.S., Israel and Kuwait, AMONDYS 45 in the U.S. and Kuwait, and VYONDYS 53 and ELEVIDYS have been approved for marketing only in the U.S. Our commercial products are also available in additional countries through our EAP. The commercial success of our products continues to depend on, and the commercial success of any future products would depend on, a number of factors attributable to one of our products or the products of our competitors, including, but not limited to:

- the effectiveness of our sales, managed markets, marketing efforts and support for our products;
- the generation and dissemination of new data analyses and the consistency of any new data with prior results, whether they support a favorable safety, efficacy and effectiveness profile of our products and any potential impact on our FDA accelerated approval status and/or FDA package insert for our products;
- the effectiveness of our ongoing commercialization activities, including negotiating and entering into any additional commercial, supply and distribution contracts, ongoing manufacturing efforts and hiring any additional personnel as needed to support commercial efforts;
- our ability to timely comply with FDA post-marketing requirements and commitments, including through successfully conducting additional studies that confirm clinical efficacy, effectiveness and safety of our products and acceptance of the same by the FDA and medical community since continued approval may be contingent upon verification of a clinical benefit in confirmatory trials, particularly in light of FDA's expanded expedited withdrawal procedures as set forth in FDORA;
- the occurrence of any side effects, adverse reactions or misuse, or any unfavorable publicity in these areas;
- the generation of evidence describing payers, patients and/or societal value of our products;
- whether we can consistently manufacture our products and product candidates at acceptable costs;
- the rate and consistency with which our products are prescribed by physicians, which depends on physicians' views on the safety, effectiveness and efficacy of our products;
- our ability to secure and maintain adequate reimbursement for our products, including the duration of the prior-authorization as well as the number and duration of re-authorization processes required for patients who initially obtained coverage by third parties, including by government payors, managed care organizations and private health insurers;
- our ability to obtain and maintain patent protection for our products, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing on the proprietary rights of third parties;

- the development, commercialization or pricing of competing products or therapies for the treatment of Duchenne, or its symptoms, and the existence of competing clinical trials;
- our ability to increase awareness of the importance of genetic testing and knowing/understanding Duchenne mutations, and identifying and addressing procedural barriers to obtaining therapy;
- our ability to remain compliant with laws and regulations that apply to us and our commercial activities;
- the actual market-size, ability to identify patients and the demographics of patients eligible for our products, which may be different than expected;
- the sufficiency of our drug supply to meet commercial and clinical demands and standards, which are negatively impacted by various factors, including when our projections on the potential number of amenable patients and their average weight are inaccurate; the potential impacts of the COVID-19 pandemic; if regulatory requirements increase our drug supply needs; if our current drug supply is destroyed or negatively impacted at our manufacturing sites, storage sites or in transit; failure to meet cGMP requirements; or if we encounter delays expanding the number of patients on our products and portions of our products' supply expire before sale;
- our ability to obtain regulatory approvals to commercialize our product candidates, and to commercialize our products in markets outside of the U.S.;
- the process leading to a patient's first infusion of our products and any future commercial products may be slower for certain patients. For example, the time to first infusion may take longer if a patient chooses to put in an intravenous port, which eases access to the vein. Delays in the process prior to first infusion could negatively impact the sales of our products, including any future gene therapy products; and
- the exercise by Roche of its option to obtain an exclusive license to commercialize one or more of our Duchenne products beyond ELEVIDYS outside of the U.S. and Roche's subsequent commercialization efforts.

In addition, the response to COVID-19 by healthcare providers has made it difficult for some patients to receive infusions or initiate treatment with our commercial products. For this and other reasons, such as delays in processing reauthorizations and modifications to program benefits by insurers, we expect that COVID-19 will reduce our revenue from commercial product sales. We experience significant fluctuations in sales of our products from period to period and, ultimately, we may never generate sufficient revenues from our products to reach or maintain profitability or sustain our anticipated levels of operations.

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

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

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

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

- issue warning letters or untitled letters;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw or alter the conditions of our marketing approval;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- suspend any ongoing clinical trials;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- refuse to approve pending applications or supplements to applications submitted by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements;
- seize or detain products, refuse to permit the import or export of products or require us to initiate a product recall; or
- refuse to allow us to enter into supply contracts, including government contracts.

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

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

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

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

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

We expect to experience pricing pressures in connection with the sale of our current and future products due to a number of factors, including current and future healthcare reforms and initiatives by government health programs and private insurers (including

managed care plans) to reduce healthcare costs, the scrutiny of pharmaceutical pricing, the ongoing debates on reducing government spending and additional legislative proposals. These healthcare reform efforts or any future legislation or regulatory actions aimed at controlling and reducing healthcare costs, including through measures designed to limit reimbursement, restrict access or impose unfavorable pricing modifications on pharmaceutical products, could impact our and our partners' ability to obtain or maintain reimbursement for our products at satisfactory levels, or at all, which could materially harm our business and financial results.

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

The downward pressure on healthcare costs in general has become intense. As a result, increasingly high barriers are being erected to the entry of new products. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our products and product candidates will be harmed. The manner and level at which reimbursement is provided for services related to our products and product candidates (e.g., for administration of our products to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and limit our ability to market or sell our products.

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

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

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid and private insurance healthcare costs, including proposed or implemented reforms involving price controls, waivers from Medicaid drug rebate law requirements, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs and the introduction of international reference pricing in the U.S. We anticipate that the Biden administration and 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, among other possible actions, implementation or modification of:

- controls on government funded reimbursement for drugs;
- caps or mandatory discounts under certain government sponsored programs;
- caps on drug reimbursement under commercial insurance;
- challenges to the pricing of drugs or limits or prohibitions on reimbursement for specific products through other means;
- reform of drug importation laws;
- delegation of decision making to state Medicaid agencies and waiver of reimbursement requirements;
- 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.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

We established a global EAP for our products in some countries where these products currently have not been approved. While we generate revenue from the distribution of these products through our EAP, we cannot predict whether historical revenues from this program will continue, whether we will be able to continue to distribute our products through our EAP, or whether revenues

will exceed revenues historically generated from sales through our EAP. Reimbursement through national EAPs may cease to be available if authorization for an EAP expires or is terminated. For example, healthcare providers in EAP jurisdictions may not be convinced that their patients benefit sufficiently from our products or alternatively, may prefer to wait until such time as our products are approved by a regulatory authority in their country before prescribing any of our products. Even if a healthcare provider is interested in obtaining access to our products for its patient through the EAP, the patient may not be able to obtain access to our products if funding for the drug is not secured.

Our business and financial results have not yet been adversely affected by the ongoing conflict between Russia and Ukraine. As our revenue from countries outside of the United States increases, our access to patients in that region through our EAP and our ability to generate revenue from commercial sales of our products in Russia or Ukraine may be adversely affected. The United States and other nations have raised the possibility of sanctions on companies that do business with Russia or its allies, including Belarus. We also may be adversely impacted by sanctions imposed on third parties with which we do business, such as third-party distributors and service providers of our EAP.

Any failure to maintain revenues from sales of eteplirsen, golodirsen or casimersen through our EAP and/or to generate revenues from commercial sales of these products exceeding historical sales due to issues under our EAP or due to global instability, like that resulting from the ongoing conflict between Russia and Ukraine, could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

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

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

Due to the nature of our products and product candidate pipeline, in addition to new chemical entity ("NCE") exclusivity and new biologic exclusivity, orphan drug exclusivity is especially important for our products that are eligible for orphan drug designation. For eligible products, we plan to rely on orphan drug exclusivity to maintain a competitive position. If we do not have adequate patent protection for our products, then the relative importance of obtaining regulatory exclusivity is even greater. While orphan status for any of our products, if granted or maintained, would provide market exclusivity for the time periods specified above upon approval, we would not be able to exclude other companies from obtaining regulatory approval of products using the same or similar active ingredient for the same indication during or beyond the exclusivity period applicable to our product on the basis of orphan drug status (e.g., seven years in the U.S.). For example, the exclusivity period for EXONDYS 51 will end in September 2023. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process. A recent decision in 2021 by the U.S. Court of Appeals for the Eleventh Circuit in *Catalyst Pharmaceuticals, Inc. vs. Becerra* regarding interpretation of the Orphan Drug Act's exclusivity provisions as applied to drugs and biologics approved for orphan indications narrower than the product's orphan designation has the potential to significantly broaden the scope of orphan exclusivity for such products. Depending on how FDA applies the Catalyst decision, it could impact our ability to obtain or seek to work around orphan exclusivity and might affect our ability to retain orphan exclusivity that the FDA previously has recognized for our products. Legislation has been introduced to amend the Orphan Drug Act in a way that may prevent these effects of the Catalyst decision, but it is unclear if or when such legislation could be enacted.

In addition, we may face risks with maintaining regulatory exclusivities for our products, and our protection may be circumvented, even if maintained. For instance, orphan drug exclusivity in the U.S. may be rescinded if (i) an alternative, competing product demonstrates clinical superiority to our product with orphan exclusivity; or (ii) we are unable to assure the availability of sufficient quantities of our orphan products to meet the needs of patients. Moreover, competitors may receive approval of different drugs or biologics for indications for which our prior approved orphan products have exclusivity. Orphan drug exclusivity in Europe 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. Thus, other companies may have received, or could receive, approval to market a product candidate that is granted orphan drug exclusivity for the same drug or similar drug and same orphan indication as any of our product candidates for which we plan to file an NDA, BLA or MAA. If that were to happen, our prior approved orphan products may face competition and any pending NDA, BLA or MAA for our product candidate for that indication may not be approved until the competing company's period of exclusivity has expired in the U.S. or the EU, as applicable. For example, in September 2021, the FDA issued guidance concerning its position on interpreting when gene therapy products would be considered the "same" or "different" for purposes of orphan drug exclusivity. The guidance states that if two gene therapy products have or use different vectors, the FDA generally intends to consider them to be "different" drugs. Further, according to the guidance, the FDA generally intends to consider vectors from the same viral group (e.g., adeno-associated virus 2 (AAV2) vs. adeno-associated virus 5 (AAV5)) to be different, when the differences between the vectors impact factors such as tropism, immune response avoidance, or potential insertional mutagenesis. However, there is considerable uncertainty as to the interpretation of these guidelines. As illustrated by this guidance, orphan drug exclusivity as applied to gene therapy products is an evolving area subject to change and interpretation by the FDA and therefore, we cannot be certain as to how the FDA will apply those rules to ELEVIDYS or our gene therapy product candidates.

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

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

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

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

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

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

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

The biotechnology and pharmaceutical industries are highly competitive and subject to significant and rapid technological change. We are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development in

areas in which our products and product candidates are aimed. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with our products or product candidates. For example, we face competition in the field of Duchenne by third parties who are developing or who had once developed: (i) exon skipping product candidates, such as Wave (notably for exons 51 and 53), Nippon Shinyaku (notably for exon 44 and exon 53, for which it has received FDA approval for its product Viltepso (viltolarsen)), Daiichi (notably for exon 45), Dyne Therapeutics pursuing antibody-oligonucleotide conjugates for exons 44, 45, 51, and 53, Avidity Biosciences pursuing antibody-oligonucleotide conjugates for exons 44, 45 and 51, PepGen (notably for exon 51), SQY Therapeutics and BioMarin (BMN-351 for exon 51); (ii) gene therapies, such as Pfizer and Solid (in partnership with Ultragenyx), and Regenxbio; (iii) gene editing, including CRISPR/Cas 9 approaches, such as Exonics Therapeutics (acquired by Vertex Pharmaceuticals), CRISPR Therapeutics, Editas Medicine, and Precision Biosciences (in partnership with Eli Lilly); (iv) other disease modifying approaches, such as PTC Therapeutics, which has a small molecule candidate, ataluren, that targets nonsense mutations; and (v) other approaches that may be palliative in nature or potentially complementary with our products and product candidates and that are or were once being developed by Santhera, Catabasis, Fibrogen, ReveraGen, Capricor Therapeutics (in partnership with Nippon Shinyaku), BioPhytis, Mallinckrodt, Antisense Therapeutics, Italfarmco, Dystrogen and Edgewise Therapeutics. Although BioMarin announced on May 31, 2016 its intent to discontinue clinical and regulatory development of drisapersen as well as its other clinical stage candidates, BMN 044, BMN 045 and BMN 053, then-currently in Phase 2 studies for distinct forms of Duchenne, it further announced its intent to continue to explore the development of next generation oligonucleotides for the treatment of Duchenne. Indeed, BioMarin has announced it is pursuing IND enabling studies for BMN-351, an oligonucleotide therapy. In addition, while Wave announced its intention to discontinue development of suvodirsen and suspend development of WVE-N531, it has announced that it commenced clinical development for its exon 53 oligonucleotide, WVE-N531.

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

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

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

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

Further, development and commercialization of ELEVIDYS and any expansion of its currently approved label, and development of our gene therapy product candidates, may compete with or supersede our current approved products, which may impact future revenues from sales of our current approved products. Our gene therapy product candidates are being developed for

potential treatment of overlapping patient populations with our current approved products, and we have not determined if our gene therapy product candidates will be used in patients in combination with our existing approved products or in separate treatment regiments.

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

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

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

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

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

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

- collaborators have significant discretion in determining the efforts and resources that they will apply to a collaboration;
- collaborators may not pursue development and commercialization of our products or product candidates based on clinical trial results, changes in their strategic focus due to the acquisition of competitive products, availability of funding, or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial, stop a clinical trial, abandon a product candidate, repeat or conduct new clinical trials, or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates, or otherwise undermine or devalue the efforts of our collaboration;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that cause the delay or termination of the research, development or commercialization of our products or product candidates, or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated and, if terminated, may eliminate our rights to commercialize certain product candidates or may result in a need for additional capital;
- failure to successfully develop the acquired or licensed drugs or technology or to achieve strategic objectives, including successfully developing and commercializing the drugs, drug candidates or technologies that we acquire or license;
- entry into markets in which we have no or limited direct prior experience or where competitors in such markets have stronger market positions;

- disruption of our ongoing business, distraction of our management and employees from other opportunities and challenges and retention of key employees;
- potential failure of the due diligence processes to identify significant problems, liabilities or other shortcomings or challenges of an acquired company, or acquired or licensed product or technology, including but not limited to, problems, liabilities or other shortcomings or challenges with respect to intellectual property, product quality, safety, accounting practices, employee, customer or third-party relations and other known and unknown liabilities;
- liability for activities of the acquired company or licensor before the acquisition or license, including intellectual property infringement claims, violations of laws, commercial disputes, tax liabilities, and other known and unknown liabilities;
- exposure to litigation or other claims in connection with, or inheritance of claims or litigation risk as a result of an acquisition or license, including but not limited to, claims from terminated employees, customers, former equity holders or other third-parties;
- difficulty in integrating the products, product candidates, technologies, business operations and personnel of an acquired asset or company; and
- difficulties in the integration of the acquired company's departments, systems, including accounting, human resource and other administrative systems, technologies, books and records, and procedures, as well as in maintaining uniform standards, controls, including internal control over financial reporting required by the Sarbanes-Oxley Act of 2002 and related procedures and policies.

For example, we will have limited influence and control over the development and commercialization activities of Roche in the territories in which it leads development and commercialization of ELEVIDYS, and if the exclusive option is exercised, in the territories in which it may lead commercialization of certain other products or product candidates. Roche's development and commercialization activities in the territories where it is the lead party may adversely impact our own efforts in the U.S. Failure by Roche to meet its obligations under the collaboration agreement, to apply sufficient efforts at developing and commercializing collaboration products, or to comply with applicable legal or regulatory requirements, may materially adversely affect our business and our results of operations. In addition, to the extent we rely on Roche to commercialize any products for which we obtain regulatory approval, we will receive less revenues than if we commercialized these products ourselves.

Even if we achieve the long-term benefits associated with strategic transactions, our expenses and short-term costs may increase materially and adversely affect our liquidity and short-term net income (loss). Future licenses or acquisitions could result in potentially dilutive issuances of our equity securities, the incurrence of debt, the creation of contingent liabilities, impairment or expenses related to goodwill, and impairment or amortization expenses related to other intangible assets, which could harm our financial condition. For example, in February 2020, we issued and sold 2,522,227 shares of common stock to Roche Finance in connection with the entry into the collaboration agreement with Roche.

Risks Related to the Development of our Product Candidates

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

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

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

- design and complexity and/or commitment of participation required in the study protocol;
- size of the patient population;
- diagnostic capabilities within patient population;
- eligibility criteria for the study in question;
- clinical supply availability;
- delays in participating site identification, qualification and subsequent activation to enroll;

- perceived risks and benefits of the product candidate under study, including as a result of adverse effects observed in similar or competing therapies;
- proximity and availability of clinical trial sites for prospective patients;
- availability of competing therapies and clinical trials;
- competition of site efforts to facilitate timely enrollment in clinical trials;
- participating site motivation;
- patient referral practices of physicians;
- activities of patient advocacy groups;
- ability to monitor patients adequately during and after treatment; and
- severity of the disease under investigation.

In particular, each of the conditions for which we plan to evaluate our product candidates are rare genetic diseases with limited patient pools from which to draw for clinical trials. Further, because newborn screening for these diseases is not widely adopted, and it can be difficult to diagnose these diseases in the absence of a genetic screen, we may have difficulty finding patients who are eligible to participate in our studies. The eligibility criteria of our clinical trials will further limit the pool of available study participants. Additionally, the process of finding and diagnosing patients may prove costly. The treating physicians in our clinical trials may also use their medical discretion in advising patients enrolled in our clinical trials to withdraw from our studies to try alternative therapies. In addition, the COVID-19 pandemic may impact patient ability and willingness to travel to clinical trial sites as a result of quarantines and other restrictions, which may negatively impact enrollment in our clinical trials.

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

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

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business.

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

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

- denial by the regulatory agencies of permission to proceed with our planned clinical trials or any other clinical trials we may initiate, or placement of a clinical trial on hold;
- delays in filing or receiving approvals of additional INDs that may be required;
- negative and/or unanticipated results from our ongoing non-clinical trials or clinical trials;

- challenges in identifying, recruiting, enrolling and retaining patients to participate in clinical trials;
- challenges with subject compliance within clinical trials;
- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the CROs/ vendors involved in the clinical trial;
- negotiate contracts and other related documents with clinical trial parties and institutional review boards, such as informed consents, CRO agreements and site agreements, which can be subject to extensive negotiations that could cause significant delays in the clinical trial process, with terms possibly varying significantly among different trial sites and CROs and possibly subjecting the Company to various risks;
- inadequate quantity or quality of supplies of a product candidate or other materials necessary to conduct clinical trials, for example as a result of delays in defining and implementing the manufacturing process for materials used in pivotal trials or for the manufacture of larger quantities or other delays or issues arising in the manufacturing of sufficient supply of finished drug product;
- difficulties obtaining institutional review board ("IRB") approval, and equivalent (Ethics Committees or ECs) approval for sites outside the U.S., to conduct a clinical trial at a prospective site or sites;
- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;
- delays or problems in analyzing data, or the need for additional analysis or data or the need to enroll additional patients;
- the occurrence of serious adverse events or unexpected drug-related side effects experienced by patients in a clinical trial or unexpected results in ongoing non-clinical trials;
- delays in validating endpoints utilized in a clinical trial;
- delays in validating outcome assessments needed in a clinical trial;
- our inability to have formal meetings with the regulatory agencies or to interact with them on a regular basis;
- our inability to satisfy the requirements of the regulatory agencies to commence clinical trials, such as developing potency assays and lot release specifications that correlate with the activity or response of the product candidate or other CMC requirements;
- the regulatory agencies disagreeing with our clinical trial design and our interpretation of data from clinical trials, or changing the requirements for approval even after the regulatory authority has reviewed and commented on the design for our clinical trials;
- reports from non-clinical or clinical testing of competing therapies that raise safety or efficacy concerns; and
- the recruitment and retention of employees, consultants or contractors with the required level of expertise.

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

Clinical development is lengthy and uncertain. Clinical trials of our novel gene therapy candidates may be delayed, including as a result of the COVID-19 pandemic, and certain programs may never advance in the clinic or may be more costly to conduct than we anticipate, any of which could have a material adverse impact on our business.

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

- the FDA, other regulators, IRBs, or ethics committees may not authorize us or our investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site for any number of reasons, including concerns regarding safety and aspects of the clinical trial design;

- we may experience delays in reaching, or fail to reach, agreement on favorable terms with prospective trial sites and prospective CROs, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- the outcome of our pre-clinical studies and our early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results;
- we may be unable to establish clinical endpoints that applicable regulatory authorities would consider clinically meaningful;
- clinical trials of any product candidates may fail to show safety or efficacy, or produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional nonclinical studies or clinical trials, or we may decide to abandon product development programs;
- differences in trial design between early-stage clinical trials and later-stage clinical trials make it difficult to extrapolate the results of earlier clinical trials to later clinical trials;
- pre-clinical and clinical data are often susceptible to varying interpretations and analyses, and many product candidates believed to have performed satisfactorily in pre-clinical studies and clinical trials have nonetheless failed to obtain marketing approval; and
- regulators may elect to impose a clinical hold, or we or our investigators, IRBs, or ethics committees may elect to suspend or terminate clinical research or trials for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable benefit risk ratio. For example, in the past we have received clinical holds from the FDA. Although these holds have generally not materially affected our development timelines, there is no assurance that any future hold would not have a material adverse effect. A clinical hold, or any of the above factors, may be out of our control and could materially impair our development timelines, expenses and results of operations.

In addition, the impact of COVID-19 has caused disruptions and may cause future delays in some of our clinical trials. Responses to COVID-19 by healthcare providers and regulatory agencies could delay the commencement of clinical trials, site initiation, protocol compliance, or the completion of clinical trials, including the completion of post-marketing requirements and commitments, slow down enrollment, and make the ongoing collection of data for patients enrolled in studies more difficult or intermittent.

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

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive pre-clinical and clinical trials, that the product candidate is safe and effective in humans. Ongoing and future pre-clinical and clinical trials of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. For example, although we believe the data for SRP-9003 and SRP-5051 collected to date are positive, the additional data we collect may not be consistent with the pre-clinical and/or early clinical data or show a safe benefit that warrants further development or pursuit of a regulatory approval for these 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. Some of our clinical trials were conducted with small patient populations and were not blinded or placebo-controlled, making it difficult to predict whether the favorable results that we observed in such trials will be repeated in larger and more advanced clinical trials. For example, our most recent announcements for SRP-9003 and SRP-5051 include: in May 2021, we announced results from the 30 mg/kg cohort of Part A of Study 5051-201 for SRP-5051; and in March 2022, we announced 24-month functional data from two clinical trial participants in the high-dose cohort, and 36-month functional data from three clinical trial participants in the low-dose cohort for SRP-9003. These data are based on small patient samples, and, given the heterogeneity of Duchenne and LGMD patients and potential lot-to-lot variability, the data may not be predictive of future results. In addition, we cannot assure that the results of additional data or data from any future trial will yield results that are consistent with the data presented, that we will be able to demonstrate the safety and efficacy of these product candidates, that later trial results will support further development, or even if such later results are favorable, that we will be able to successfully complete the development of, obtain accelerated, conditional or standard regulatory approval for, or successfully commercialize any of such product candidates. Similarly, we cannot provide assurances that data from our ongoing and planned studies with respect to our commercially approved products and product candidates will be positive and consistent or that the interpretation by regulators, such as the FDA or EMA, of the data we collect for our products or product candidates will be consistent with our interpretations.

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

Our product candidates may cause undesirable side effects. In addition to side effects caused by our product candidates, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur in our trials, we may decide, or the FDA, the EMA or other regulatory authorities could order us, to halt, delay or amend pre-clinical development or clinical development of our product candidates or we may be unable to receive regulatory approval of our product candidates for any or all targeted indications. For example, FDA placed Study 5051-201 on clinical hold in June 2022 following a serious adverse event of hypomagnesemia, which was lifted in August 2022. Even if we are able to demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates and may harm our business, financial condition and prospects significantly.

If there are significant delays in obtaining or 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. Of the large number of drugs in development in the biopharmaceutical industry, only a small percentage result in the submission of a marketing application to the FDA or an MAA to the EMA and even fewer are approved for commercialization.

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

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

- We may not have the resources required to meet regulatory requirements and successfully navigate what is generally a lengthy, expensive and extensive approval process for commercialization of drug product candidates.

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

Even if our product candidates demonstrate safety and efficacy in clinical studies, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory advisory group or authority recommends non-approval or restrictions on approval.

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

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

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

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

Regulatory requirements governing gene therapy products have evolved and may continue to change in the future. Within the FDA, the Center for Biologics Evaluation and Research (“CBER”) regulates gene therapy products. Within the CBER, the review of gene therapy and related products is consolidated in the Office of Cellular, Tissue and Gene Therapies, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. The CBER works closely with the National Institutes of Health (the “NIH”). The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols. For example, on January 28, 2020, the FDA issued final guidance documents that updated draft guidance documents that were originally released in July 2018 to reflect recent advances in the field, and to set forth the

framework for the development, review and approval of gene therapies. These final guidance documents pertain to the development of gene therapies for the treatment of specific disease categories, including rare diseases, and to manufacturing and long-term follow up issues relevant to gene therapy, among other topics. The FDA also issued a new guidance document in September 2021 describing the FDA's approach for determining whether two gene therapy products were the same or different for the purpose of assessing orphan drug exclusivity, as well as a draft guidance document in March 2022 on human gene therapy product incorporating human genome editing. In addition, the FDA can put an IND on hold if the information in an IND is not sufficient to assess the risks in pediatric patients.

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

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

Because we are developing product candidates for the treatment of certain diseases in which there is little clinical experience and we are using new endpoints or methodologies, there is increased risk that the FDA, the EMA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze.

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

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

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

We may seek fast track, breakthrough therapy designation, RMAT designation, PRIME scheme access or priority review designation for our product candidates if supported by the results of clinical trials. A fast track product designation is designed to facilitate the clinical development and expedite the review of drugs intended to treat a serious or life-threatening condition which demonstrate the potential to address an unmet medical need. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. A RMAT designation is designed to accelerate approval for regenerative advanced therapies such as our gene therapy product candidates. Priority review designation is intended to speed the FDA marketing application review timeframe for drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. PRIME is a scheme provided by the EMA to enhance support for the development of medicines that target an unmet medical need.

For drugs and biologics that have been designated as fast track products or breakthrough therapies, or granted access to the PRIME scheme, interaction and communication between the regulatory agency and the sponsor of the trial can help to identify the most efficient path for clinical development. Sponsors of drugs with fast track products or breakthrough therapies may also be able to submit marketing applications on a rolling basis, meaning that the FDA may review portions of a marketing application before the sponsor submits the complete application to the FDA, if the sponsor pays the user fee upon submission of the first portion of the marketing application. For products that receive a priority review designation, the FDA's marketing application review goal is shortened to six months, as opposed to ten months under standard review. This review goal is based on the date the FDA accepts the marketing application for review, this application validation period typically adds approximately two months to the timeline for review and decision from the date of submission. RMAT designations will accelerate approval and will include all the benefits of fast track and breakthrough therapy designations, including early interactions with the FDA, but the exact mechanisms have not yet been announced by FDA.

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

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

Our pipeline includes more than 40 programs in various stages of development for a broad range of diseases and disorders. We plan to expand our pipeline through internal research and development and through strategic transactions. Because we have limited resources, we may not be able to advance all of our programs. We may also forego or delay pursuit of opportunities with certain programs or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

Risks Related to Third Parties

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

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

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

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

- not provide us with accurate or timely information regarding their inventories, the number of patients who are using our products or serious adverse events and/or product complaints regarding our products;
- not effectively sell or support our products;

- reduce or discontinue their efforts to sell or support our products;
- not devote the resources necessary to sell our products in the volumes and within the time frame we expect;
- be unable to satisfy financial obligations to us or others; or
- cease operations.

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

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

Furthermore, a significant outbreak of COVID-19 at one of our third-party logistics, distribution, or specialty pharmacy sites could lead to a delay in the commercial or pre-commercial shipments of our products to patients and hospitals.

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

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

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

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

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

Our reliance on third-party collaborators requires us to disclose our proprietary information to these parties, which could increase the risk that a competitor will discover this information or that this information will be misappropriated or disclosed without

our intent to do so. If any of these events were to occur, then our ability to obtain patent protection or other intellectual property rights could be irrevocably jeopardized, and costly, distracting litigation could ensue. Furthermore, if these third parties cease to continue operations and we are not able to quickly find a replacement provider or we lose information or items associated with our products or product candidates, our development programs may be delayed. Although we carefully manage our relationships with our third-party collaborators and CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

Risks Related to Manufacturing

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

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

In addition, the process for adding new manufacturing capacity is lengthy and often causes delays in development efforts. Any interruption of the development or operation of those facilities due to, among other reasons, events such as the ongoing COVID-19 pandemic, order delays for equipment or materials, equipment malfunctions, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters, such as earthquakes or fires, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in supply of our products, product candidates or materials. Any delay or interruption in the supply of finished products could hinder our ability to distribute our products to meet commercial demand or execute our commercialization plans on the timing that we expect, which could result in the loss of potential revenues, adversely affect our ability to gain market acceptance, or otherwise adversely affect our business, financial condition and prospects.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

Our contract manufacturers are required to produce our materials, APIs and drug products under cGMP. We and our contract manufacturers are subject to periodic inspections by the FDA, EMA and corresponding state and foreign authorities to ensure strict

compliance with cGMP and other applicable government regulations. In addition, before we can begin to commercially manufacture our product candidates in third-party or our own facilities, we must obtain regulatory approval from the FDA, which includes a review of the manufacturing process and facility. A manufacturing authorization also must be obtained from the appropriate EU regulatory authorities and may be required by other foreign regulatory authorities. The timeframe required to obtain such approval or authorization is uncertain. In order to obtain approval, we need to demonstrate that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. In complying with cGMP, we are obligated to expend time, money and effort in production, record keeping and quality control to seek to assure that the product meets applicable specifications and other requirements.

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

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

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

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

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

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

Risks Related to our Intellectual Property

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

We currently directly hold various issued patents and patent applications, or have exclusive license or option rights to issued patents and patent applications, in each case in the U.S. as well as other countries that protect our products, product candidates and platform technologies. We anticipate filing additional patent applications both in the U.S. and in other countries. Our success will depend, in significant part, on our ability to obtain, maintain and defend our U.S. and foreign patents covering our products, product candidates and platform technologies as well as preserving our trade secrets for these assets. The patent process is subject to numerous risks and uncertainties, and we can provide no assurance that we will be successful in obtaining, maintaining, or defending our patents. Even when our patent claims are allowed, the claims may not issue, or in the event of issuance, may not be sufficient to protect our products, product candidates or platform technologies or may be challenged in post-grant proceedings by third parties.

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

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

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

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

The patent landscape is continually evolving, and we may be able to assert that certain activities engaged in by third parties infringe our current or future patent rights. There has been, and we believe that there will continue to be, significant litigation in the

biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights. As such, the patents and patent applications that we own, license, have optioned, and rely on for exclusivity for our product candidates may be challenged.

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

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

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 that may also affect patent litigation. The USPTO has issued regulations and procedures to govern administration of the Leahy-Smith Act. In view of the long timelines for interpreting legal provisions in the court system and the evolving nature of our laws, 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 Patent Trial and Appeal Board ("PTAB") seeking to challenge some or all of the claims in any of our patents through an inter partes review or other post-grant proceedings. Should the PTAB or the USPTO Director institute an inter partes review or other proceedings and the PTAB decide that some or all of the claims in the challenged patent are unpatentable, unenforceable, or invalid, such a decision, if upheld on appeal, could have a material adverse effect on our business and financial condition.

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

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

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

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

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

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

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

Risks Related to our Business Operations

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

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

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

The number and complexity of both federal and state laws continues to increase, and additional governmental resources are being used to enforce these laws and to prosecute companies and individuals who are believed to be violating them. We anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of government investigations and enforcement actions.

We have implemented a compliance program, which is based on industry best practices and is designed to ensure that our activities comply with all applicable laws, regulations and industry standards. While our compliance program is intended to detect and

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

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

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

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

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

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

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

The COVID-19 pandemic has resulted, and may continue to result in disruptions to our commercialization, clinical trials, manufacturing and other business operations, which could have a material adverse effect on our business, financial condition, operating results, cash flows and prospects.

The COVID-19 pandemic has presented a substantial public health and economic challenge around the world. The rapid spread of COVID-19 has led to the implementation of various responses, including government-imposed quarantines, shelter-in-place mandates, sweeping restrictions on travel, mandatory shutdowns for non-essential businesses, requirements regarding social distancing, and other public health safety measures, as well as reported adverse impacts on healthcare resources, facilities and providers across the United States and in other countries. In response to the pandemic, healthcare providers have, and may need to further, reallocate resources, such as physicians, staff, hospital beds, and intensive care unit facilities, as they prioritize limited resources and personnel capacity to focus on the treatment of patients with COVID-19 and implement limitations on access to hospitals and other medical institutions due to concerns about the spread of COVID-19 in such settings. These responses may be extended by the duration of the outbreak, periodic spikes in infection rates due to new strains of the virus, new information that will emerge concerning the severity of the coronavirus and the actions to contain the coronavirus or treat its impact. These actions have and may continue to negatively impact commercialization, clinical trials, manufacturing and other business operations, including:

- **Commercial:** The response to COVID-19 by healthcare providers has made it difficult for some patients, especially those dependent on a hospital setting, to receive infusions or initiate treatment with our commercial products. In addition, as a result of the pandemic, some patients may choose to delay or stop treatment to avoid a visit to a hospital or a visit of a third party in their homes to minimize the risk of infection. In some cases, at home infusions have been delayed due to outbreaks of COVID-19 among trained personnel and staffing shortages at times during periodic spikes in infection rates. These challenges may continue for the duration of the COVID-19 pandemic, which is uncertain, and are expected to reduce our revenue and cash flows.
- **Clinical trials:** The impact of COVID-19 has caused disruptions and may cause delays in some of our clinical trials. Missing data could undermine data integrity and probability of success. The response to COVID-19 by healthcare providers and regulatory agencies could delay the commencement of trials, site initiation, compliance in the trials, the completion of trials, including the completion of post-marketing requirements and commitments, slow down enrollment, and make the ongoing collection of data for patients enrolled in studies more difficult or intermittent. In addition, as COVID-19 continues to spread, some participants and clinical investigators may be unable or unwilling to comply with clinical trial protocols. For example, quarantines or other travel limitations (whether voluntary or required) were implemented in many countries during the pandemic, and may impede participant movement, affect sponsor access to study sites, or interrupt healthcare services, which may negatively impact the execution of clinical trials. Significant delays or disruptions to our clinical trials could adversely affect our ability to timely initiate studies, conduct successful studies, obtain or maintain regulatory approvals, or commercialize our product candidates.
- **Operations:** Remote working increases our vulnerability to cyber security breaches. Further, if the spread of the COVID-19 pandemic continues and our operations are adversely impacted, including due to an outbreak in a facility, we risk a delay, default and/or nonperformance under existing agreements.

Any of the foregoing factors could have a material adverse impact on our business, financial condition, operating results, cash flows and prospects. The extent to which COVID-19 impacts our operations and those of our third-party partners will depend on future developments, which are highly uncertain and cannot be predicted with confidence, including the duration of the pandemic, additional or modified government actions, new information which emerges concerning the severity of COVID-19 and the actions taken to contain the virus or treat its impact, among others. In particular, the speed of the continued spread of COVID-19 globally, and the magnitude of interventions to contain the spread of the virus, will determine the impact of the pandemic on our operations.

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

We have incurred substantial losses during our history and expect to incur more as we pursue our business strategy. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset a portion of future taxable income, if any, subject to expiration of such carryforwards in the case of carryforwards generated prior to January 1, 2018. In general, under Section 382 of the Internal Revenue Code, a corporation that undergoes an “ownership change” is subject to limitations on its ability to utilize its pre-change net operating losses and certain other tax assets (including R&D tax credits) to offset future taxable income. In general, an ownership change occurs if the aggregate stock ownership of certain stockholders increases by more than 50 percentage points over such stockholders’ lowest percentage ownership during the testing period, which is generally three years. An ownership change could limit our ability to utilize our net operating loss and tax credit carryforwards for taxable years including or following such “ownership change.” Such limitations may result in expiration of a portion of the net operating loss carryforwards incurred prior to 2018 before utilization and may be substantial. If such change has occurred or does occur, the tax benefits related to the net operating loss carryforwards and certain other tax assets may be limited or lost. Moreover, proposed U.S. Treasury Regulations promulgated under Section 382 of the Code could, if finalized, significantly impact a corporation’s ability to use its pre-change net operating loss

carryforwards or other attributes following an ownership change. Limitations imposed on the ability to use net operating losses and tax credits to offset future taxable income could require us to pay U.S. federal income taxes earlier than we estimated or than would have otherwise been required if such limitations were not in effect and could cause such net operating losses and tax credits to expire unused, in each case reducing or eliminating the benefit of such net operating losses and tax credits and potentially adversely affecting our financial position. Similar rules and limitations may apply for state income tax purposes. At the state level, there may also be periods during which the use of net operating loss carryforwards or other attributes is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed. These net operating losses have been fully offset by a valuation allowance due to uncertainties surrounding our ability to realize these tax benefits.

On August 16, 2022, the Inflation Reduction Act of 2022, which includes changes to the U.S. federal taxation of corporations, was enacted into law. The Inflation Reduction Act among other things implements a corporate book minimum tax ("BMT") 15% rate that could apply to consolidated groups of companies with adjusted financial statement income in excess of $1.0 billion over a three-year period. The BMT has various limitations, including a more restrictive limit on availability of net operating loss carryforwards, which if applied to us, could impact its cash tax liability and ability to utilize tax attributes.

In addition, many of the jurisdictions in which we operate have or are expected to adopt changes to tax laws as a result of the Base Erosion and Profit Shifting final proposals from the Organization for Economic Co-operation and Development and specific country anti-avoidance initiatives. In addition, the current proposal of the BMT may result in increases in tax imposed by non-U.S. jurisdictions. Such tax law changes and anti-avoidance initiatives increase uncertainty and may adversely affect our tax provision, cash tax liability and effective tax rate.

We are winding down our expired U.S. government contracts, and the U.S. government may deny payment of some or all of the currently outstanding amounts owed to us. In addition, further development of our infectious disease programs may be limited 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 infectious disease development programs. These contracts expired and we are currently involved in contract close-out activities. The U.S. government has the right to perform additional audits prior to making final payment of costs and fees. If we are not able to adequately support costs incurred or other government requirements, the government may deny payment of some or all of the currently outstanding amounts owed to us. In addition, 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.

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

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

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

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

The competition for qualified personnel in the biotechnology field is intense, and our future success depends upon our ability to attract, retain, motivate and support such personnel. The COVID-19 pandemic has exacerbated workforce competition and workforce shortages. In order to develop and commercialize our products successfully, we will be required to retain key management and scientific employees. In certain instances, we may also need to expand or replace our workforce and our management ranks. In addition, we rely on certain consultants and advisors, including scientific and clinical advisors, to assist us in the formulation and advancement of our research and development programs. Our consultants and advisors may be employed by other entities or have

commitments under consulting or advisory contracts with third parties that limit their availability to us, or both. If we are unable to attract, assimilate or retain such key personnel, our ability to advance our programs would be adversely affected.

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

Risks Related to our Financial Condition and Capital Requirements

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

We incurred an operating loss of $271.6 million for the six months ended June 30, 2023. Our accumulated deficit was $4.5 billion as of June 30, 2023. Although we currently have four commercially approved products in the U.S., we believe that it will take us some time to attain profitability and positive cash flow from operations. Since our products and product candidates target small patient populations, the per-patient drug pricing must be high in order to recover our development and manufacturing costs, fund adequate patient support programs, fund additional research and achieve profitability. We may be unable to maintain or obtain sufficient sales volumes at a price high enough to justify our product development efforts and our sales, marketing and manufacturing expenses.

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

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

As a result, we expect to continue to incur significant operating losses at least through 2023. Because of the numerous risks and uncertainties associated with developing biopharmaceutical products, we are unable to predict the extent of any future losses or when, or if, we will become profitable.

We may need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

We may require additional capital from time to time in the future in order to meet FDA post-marketing approval requirements and market and sell our products as well as to continue the development of product candidates in our pipeline, to prepare for potential commercialization of additional 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.

While we are currently well capitalized, we may use available capital resources sooner than we expect under our current operating plan. In addition, our operating plan may change. We may need or choose to seek additional funds sooner than planned, through equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances, funded research and development arrangements and licensing arrangements or a combination of these approaches. In any event, we expect to require additional capital to expand future development efforts, obtain regulatory approval for, and to commercialize, our product candidates. Raising funds in the current economic environment may present additional challenges. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or in light of specific strategic considerations.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. In the event we receive negative data from our key clinical programs or encounter other major setbacks in our development, manufacturing or regulatory activities or in our commercialization efforts, our stock price is likely to decline, which would make a future financing more difficult. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders. The issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities may dilute all of our stockholders. The incurrence of indebtedness may result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners 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.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product, if approved, or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations.

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

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

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

Our consolidated financial statements and condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the U.S. The preparation of these statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. Such estimates and judgments include revenue recognition, inventory, valuation of stock-based awards, research and development expenses and income tax. We base our estimates on historical experience, facts and

circumstances known to us and on various other assumptions that we believe to be reasonable under the circumstances. We cannot provide assurances, however, that our estimates, or the assumptions underlying them, will not change over time or otherwise prove inaccurate. If this is the case, we may be required to restate our consolidated financial statements or condensed consolidated financial statements, which could, in turn, subject us to securities class action litigation. Defending against such potential litigation relating to a restatement of our consolidated financial statements or condensed consolidated financial statements would be expensive and would require significant attention and resources of our management. Moreover, our insurance to cover our obligations with respect to the ultimate resolution of any such litigation may be inadequate. As a result of these factors, any such potential litigation could have a material adverse effect on our financial results and cause our stock price to decline, which could in turn subject us to securities class action litigation.

Risks Related to Our Common Stock

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

The market prices for and trading volumes of securities of biotechnology companies, including our securities, 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, as of the date of this report, our stock has increased as much as 31% in a single day or decreased as much as 18% in a single day. The market has from time to time experienced significant price and volume fluctuations unrelated to the operating performance of particular companies. The market price of our common stock may fluctuate significantly due to a variety of factors, including but not limited to:

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

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

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

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

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

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

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

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

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

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

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

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

Currently, our Amended and Restated Certificate of Incorporation authorizes the issuance of up to 198.0 million shares of common stock. As of June 30, 2023, there were approximately 93.3 million shares of common stock outstanding and outstanding awards to purchase 12.1 million shares of common stock under various incentive stock plans. Additionally, as of June 30, 2023, there were approximately 2.6 million shares of common stock available for future issuance under our 2018 Equity Incentive Plan, approximately 0.1 million shares of common stock available for issuance under our Amended and Restated 2013 Employee Stock Purchase Plan, and approximately 0.7 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan.

We may issue additional shares to grant equity awards to our employees, officers, directors and consultants under our 2018 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. We may also issue additional common stock and warrants from time to time to finance our operations and in connection with strategic transactions, such as acquisitions and licensing. For example, in February 2020, we issued and sold 2,522,227 shares of common stock to Roche Finance in connection with the entry into the collaboration agreement with Roche.

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

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

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

Risks Related to Our Convertible Senior Notes

Servicing our 1.50% notes due 2024 (the "2024 Notes") and 1.25% notes due 2027 (the "2027 Notes", and together with the 2024 Notes, the "Notes") requires a significant amount of cash, and we may not have sufficient cash flow to pay our debt.

In 2017, we issued $570.0 million aggregate principal amount of Notes, pursuant to that certain indenture, dated as of November 14, 2019, between us, as issuer, and U.S. Bank National Association, as trustee. In September 2022, we issued $1,150.0 million aggregate principal amount of 2027 Notes, pursuant to that certain indenture dated as of September 16, 2022, between us, as issuer, and U.S. Bank National Association, as trustee, including $20.0 million of 2027 Notes issued to the Michael A. Chambers Living Trust in a private placement. In September 2022, we entered into separate, privately negotiated transactions to repurchase a portion of the outstanding 2024 Notes and, in March 2023, we entered into separate, privately negotiated exchange agreements with holders of $313.5 million in aggregate principal value of outstanding 2024 Notes pursuant to which these 2024 Notes were exchanged

for shares of our common stock. Our ability to make scheduled payments of the principal of, to pay interest on, or to refinance our indebtedness, including the Notes, depends on our future performance, which is subject to many factors, including, economic, financial, competitive and other, beyond our control. We do not expect our business to be able to generate cash flow from operations in the foreseeable future, sufficient to service our debt and make necessary capital expenditures and we may therefore be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be onerous or highly dilutive. Our ability to refinance the remaining outstanding 2024 Notes, which are non-callable and mature in 2024, and the 2027 Notes, which mature in 2027, will depend on the capital markets and our financial condition at such times. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations, and limit our flexibility in planning for and reacting to changes in our business.

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

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

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

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

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

General Risks

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

Our results of operations could be harmed by general conditions in the global economy and in the global financial markets. A severe or prolonged economic downturn, including the impact of increased interest rates and inflation (such as the recent rise in inflation in the United States), could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could strain our manufacturers, possibly resulting in manufacturing disruption, or cause delays in payments for our services by third-party payors or

our future collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could harm our business.

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

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

Violation of the General Data Protection Regulation could subject us to significant fines.

The GDPR increases our obligations with respect to clinical trials conducted in the member states of the EEA by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR increases the scrutiny that clinical trial sites located in the EEA should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the U.S. The GDPR imposes substantial fines for breaches of data protection requirements, which can be up to four percent of global revenue or 20 million Euros, whichever is greater, and it also confers a private right of action on data subjects for breaches of data protection requirements. Compliance with these directives will be a rigorous and time-intensive process that may increase our cost of doing business, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation and reputational harm in connection with our European activities.

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

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

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

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

- the inability to obtain necessary foreign regulatory or pricing approvals of products in a timely manner;
- uncertainties regarding the collectability of accounts receivable;
- fluctuations in foreign currency exchange rates that may adversely impact our revenues, net income and value of certain of our investments;
- difficulties in staffing and managing international operations;
- the imposition of governmental controls;
- less favorable intellectual property or other applicable laws;
- increasingly complex standards for complying with foreign laws and regulations that may differ substantially from country to country and may conflict with corresponding U.S. laws and regulations;

- the far-reaching anti-bribery and anti-corruption legislation in the U.K., including the U.K. Bribery Act 2010, and elsewhere and escalation of investigations and prosecutions pursuant to such laws;
- compliance with complex import and export control laws;
- restrictions on direct investments by foreign entities and trade restrictions; and
- changes in tax laws and tariffs.

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

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any 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 the patients using our commercially approved products, clinical trial participants and employees. Similarly, our third-party providers possess certain of our sensitive data. The secure maintenance of this information is critical to our operations and business strategy. Our ongoing operating activities also depend on functioning computer systems. Despite our security measures, our information technology and infrastructure are subject to attacks or breaches. Any such breach could result in a material compromise of our networks, and the information stored there could be accessed, publicly disclosed, lost, stolen, or rendered, permanently or temporarily, inaccessible. Furthermore, we may not promptly discover a system intrusion. Attacks could have a material impact on our business, operations or financial results. Any such access, disclosure or other loss of information, including our data being breached at third party providers, could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations and damage our reputation, which could adversely affect our business. We also may need to pay "ransomware" to re-access our systems.

In addition, privacy and data protection laws may be interpreted and applied differently from country to country and may create inconsistent or conflicting requirements, which increase the costs incurred by us in complying with such laws. The European Union's GDPR, which greatly increases the jurisdictional reach of European Union law and became effective in May 2018, adds a broad array of requirements for handling personal data including the public disclosure of significant data breaches, and imposes substantial penalties for non-compliance of up to the greater of €20 million or 4% of global annual revenue for the preceding financial year. Our efforts to comply with GDPR and other privacy and data protection laws imposes significant costs and challenges that are likely to increase over time, and we are exposed to substantial penalties or litigation related to violations of existing or future data privacy laws and regulations.

Additionally, the CCPA, which became effective January 1, 2020, substantially expands privacy obligations of many businesses. The CCPA requires new disclosures to California consumers, imposes new rules for collecting or using information about minors, and affords consumers new abilities, such as the right to know whether the data is sold or disclosed and to whom, the right to request that a company delete personal information collected, the right to opt-out of the sale of personal information and the right to non-discrimination in terms of price or service when a consumer exercises a privacy right. Failure to comply with these regulations is subject to civil sanctions, including fines and penalties. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. Moreover, a newly passed ballot initiative, the California Privacy Rights Act ("CPRA"), which took effect on January 1, 2023, expands on the CCPA, creating new consumer rights and protections, including the right to correct personal information, the right to opt out of the use of personal information in automated decision making, the right to opt out of "sharing" consumer's personal information for cross-context behavioral advertising, and the right to restrict use of and disclosure of sensitive personal information, including geolocation data to third parties. We will need to evaluate and potentially update our privacy program to seek to comply with the CPRA and will incur additional costs and expenses in our effort to comply.

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.

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

Social media is increasingly being used to communicate about our products, technologies and programs, and the diseases our product and product candidates are designed to treat. Social media practices in the biopharmaceutical industry continue to evolve and regulations relating to such use are not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media channels to comment on the effectiveness of a product or to report an alleged adverse event. When such disclosures occur, there is a risk that we fail to monitor and comply with applicable adverse event reporting obligations or we may not be able to defend ourselves or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our product and/or product candidates. There is also a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us on any social networking website. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face overly restrictive regulatory actions or incur other harm to our business.

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

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

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

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

During the three months ended June 30, 2023, no director or officer of the Company adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement" as each term is defined in Item 408(a) of Regulation S-K.

Item 6. Exhibits.

The exhibits listed on the Exhibit Index immediately preceding such exhibits, which is incorporated herein by reference, are filed or furnished as part of this Quarterly Report on Form 10-Q.

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
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	Certificate of Amendment to the Amended and Restated Certificate of Incorporation of Sarepta Therapeutics, Inc.	8-K	001-14895	3.1	6/8/20	
3.4	Amended and Restated Bylaws.	8-K	001-14895	3.1	9/25/14	
3.5	Amendment No. 1 to the Amended and Restated Bylaws.	8-K	001-14895	3.1	1/13/20	
3.6	Second Amended and Restated Bylaws	8-K	001-14895	3.1	12/13/22	
10.1*	Amended and Restated Lead DMD Product Manufacturing and Supply Agreement between Catalent Maryland, Inc. and Sarepta Therapeutics Three, LLC					X
10.2*	Exclusive License Agreement between the Research Institute at Nationwide Children's Hospital and Sarepta Therapeutics, Inc., dated October 8, 2018					X
10.3*	First Amendment, dated May 29, 2019, to the Exclusive License Agreement between Research Institute at Nationwide Children's Hospital and Sarepta Therapeutics, Inc.					X
10.4	Second Amendment, dated July 11, 2023, to the Exclusive License Agreement between Research Institute at Nationwide Children's Hospital and Sarepta Therapeutics, Inc.					X
10.5	First Amendment to the Amended and Restated Lead DMD Product Manufacturing & Supply Agreement between Catalent Maryland, Inc. and Sarepta Therapeutics Three, LLC					X
31.1	Certification of the Company's Principal Executive Officer, Douglas S. Ingram, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company's Principal Financial and Accounting Officer, Ian M. Estepan, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1**	Certification of the Company's Principal Executive Officer, Douglas S. Ingram, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2**	Certification of the Company's Principal Financial and Accounting Officer, Ian M. Estepan, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	Inline eXtensible Business Reporting Language (XBRL) Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.					X
101.SCH	Inline XBRL Taxonomy Extension Schema Document.					X
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document.					X

101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document.	X
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)	X

† Indicates management contract or compensatory plan, contract or arrangement.

* Certain identified information has been excluded from the exhibit.

** The Certifications attached as Exhibits 32.1 and 32.2 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the SEC and are not to be incorporated by reference into any filings of Sarepta Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Form 10-Q, irrespective of any general incorporation language contained in such filing.

SIGNATURES

Pursuant to the requirements 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.

SAREPTA THERAPEUTICS, INC.
(Registrant)

Date: August 2, 2023

By: /s/ DOUGLAS S. INGRAM
Douglas S. Ingram
President and Chief Executive Officer
(Principal Executive Officer)

Date: August 2, 2023

By: /s/ IAN M. ESTEPAN
Ian M. Estepan
Executive Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

[**] = Identified information has been excluded from this exhibit because it is both (i) information that the Company customarily and actually treats as private or confidential and (ii) is not material.

Exhibit 10.1

[**] = Identified information has been excluded from this exhibit because it is both (i) information that the Company customarily and actually treats as private or confidential and (ii) is not material

AMENDED & RESTATED

LEAD DMD PRODUCT MANUFACTURING & SUPPLY AGREEMENT

by and between

SAREPTA THERAPEUTICS THREE, LLC

and

CATALENT MARYLAND, INC.

Dated as of November 28, 2022

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AMENDED AND RESTATED
LEAD DMD PRODUCT MANUFACTURING & SUPPLY AGREEMENT

THIS AMENDED AND RESTATED LEAD DMD PRODUCT MANUFACTURING & SUPPLY AGREEMENT (this "**Agreement**"), dated as of the 28th day of November, 2022 (the "**Effective Date**"), is entered into by and among **SAREPTA THERAPEUTICS, INC.**, a corporation organized and existing under the Laws of Delaware and having a place of business at 215 First Street, Boston, Massachusetts 02142, ("**Sarepta**"), and **CATALENT MARYLAND, INC.** (formerly **PARAGON BIOSERVICES, INC.**), a corporation organized and existing under the Laws of Delaware and having a place of business at 801 West Baltimore Street, Suite 302, Baltimore, Maryland 21201 ("**Catalent**"). Sarepta and Catalent are sometimes referred to herein, individually, as a "**Party**" and, collectively, as the "**Parties**."

RECITALS

WHEREAS, Sarepta possesses expertise in the pharmaceutical research, development and commercialization of human therapeutics and has a microdystrophin gene therapy drug targeting Duchenne muscular dystrophy (hereinafter referred to as the "**Lead DMD Product**", as further defined herein);

WHEREAS, Sarepta is interested in securing commercial scale manufacturing capacity for the Lead DMD Product and other drugs of which Sarepta or its designated Affiliates or Strategic Partners may later contract with Catalent for clinical or commercial supply (collectively, the Lead DMD Product and other Sarepta gene therapy drugs that are Manufactured by Catalent pursuant to a Sarepta Supply Agreement are referred to herein as the "**Sarepta Drug Products**");

WHEREAS, Catalent has process development, manufacturing, and related services experience and expertise, and operates facilities for the development and manufacturing of biopharmaceuticals, including a commercial scale biomanufacturing facility located at the [**] Facility;

WHEREAS, on October 8, 2018, the Parties executed a Collaboration Agreement (the "**Original Collaboration Agreement**") under which Catalent committed to provide Sarepta with dedicated biomanufacturing space at the [**] Facility and, subject to future agreements, to perform certain services and clinical and commercial supply of the Sarepta Drug Products;

WHEREAS, the Original Collaboration Agreement established, among other items, (1) the terms and conditions regarding the governance of the dedicated clean room suites for the Manufacture of the Sarepta Drug Products (collectively, the "**Dedicated Clean Room Collaboration**"), and [**];

WHEREAS, on February 22, 2019 (the "**Original Effective Date**"), the Parties executed a Manufacturing and Supply Agreement for the Lead DMD Product (the "**Original Lead DMD Agreement**"), which set forth the terms and conditions as to (i) the technology transfer and clinical supply Manufacturing of the Lead DMD Product Bulk Drug Substance and Drug Product and (ii) the commercial Manufacturing of the Lead DMD Product Bulk Drug Substance and Drug Product;

WHEREAS, on May 20, 2019, Catalent Pharma Solutions, Inc., ("**CPS**") a leading global provider of advanced delivery technologies and development solutions for drugs, biologics, and consumer health products, acquired Paragon Bioservices, Inc., which is now Catalent, a subsidiary of CPS (the "**Acquisition**");

WHEREAS, on July 24, 2019, the Parties executed the first amendment to the Original Lead DMD Agreement to (i) add two additional scopes of work for process development and analytical testing involving the Lead DMD Product, attached thereto as Exhibits B-1 and B-2, respectively (the "**Process Development-Focused SOWs**"), and (ii) amend certain defined terms;

WHEREAS, on September 11, 2019 Sarepta exercised its option to take the Optional Clean Rooms (as originally defined in the Original Collaboration Agreement) and, consequently, Catalent had dedicated four clean room suites in the [**] Facility for the Manufacture of Sarepta Drug Products, two of which were maintained as dedicated to Sarepta by the Letter Agreement between the Parties of March 23, 2021 (the two suites dedicated to Sarepta, the "**Sarepta Clean Room Suites**," referred to as Suites 3 and 4, as further defined herein);

WHEREAS, the Parties contemplate that the Dedicated Clean Room Collaboration will lead to clinical and commercial supply agreements for additional products that will be negotiated and executed by the Parties, subject to the capacity of the Sarepta Clean Room Suites (the Lead DMD Agreement and such additional supply agreements are each a "**Sarepta Supply Agreement**" and collectively the "**Sarepta Supply Agreements**");

WHEREAS, contemporaneous with the execution of this Agreement, the Parties executed an Amended and Restated Collaboration Agreement (the "**Amended and Restated Collaboration Agreement**") to reflect the Parties understanding as to (i) the Parties' acknowledgment of CPS, as the acquirer of Paragon Bioservices, Inc., and the need to amend certain terms and conditions of the Original Collaboration Agreement to reflect the Acquisition, and (ii) make such other changes deemed necessary or beneficial by the Parties;

WHEREAS, the Parties intend for this Agreement to replace and supersede the Original Lead DMD Agreement, as amended, in its entirety to reflect the Parties understanding as to (i) the rights, obligations, terms, and conditions among the Parties with respect to the Sarepta Clean Room Suites pursuant to the Dedicated Clean Room Collaboration, (ii) the Parties acknowledgment of CPS, as the acquirer of Paragon Bioservices, Inc., and (iii) the need to adjust the terms and conditions of the Original Lead DMD Agreement to reflect the Acquisition and to make other adjustments deemed necessary or beneficial by the Parties.

NOW, THEREFORE, in consideration of the foregoing and the representations, warranties, covenants, agreements and provisions set forth herein, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, and intending to be legally bound hereby, the Parties agree as follows:

ARTICLE I
DEFINITIONS

1.1. Definitions. All capitalized terms used herein, including in the Exhibits and Schedules hereto, shall have the meanings specified in **Exhibit A** attached hereto or elsewhere in this Agreement, as applicable, unless otherwise specified.

ARTICLE II
FACILITY DESIGN, CONSTRUCTION, VALIDATION AND OPERATION

2.1. [**] Facility Design, Construction and Validation. Under the Amended and Restated Collaboration Agreement, Catalent is responsible for the design, construction and validation of the Sarepta Clean Room Suites at the [**] Facility, including purchasing and installing the equipment to be used in the Sarepta Clean Room Suites in accordance with the designs provided by Catalent and agreed to by Sarepta. In addition, under the Amended and Restated Collaboration Agreement, Catalent is responsible for validating and maintaining the Sarepta Clean Room Suites, as it relates to the Manufacture of Lead DMD Product according to Sarepta's process, in accordance with cGMPs and the Quality Agreement with Sarepta such that Sarepta is able to seek Regulatory Approval for use of the [**] Facility in connection with the filing of a BLA for the Lead DMD Product. Catalent shall work with Sarepta to assure that the design of the [**] Facility, the equipment used and other aspects of the [**] Facility are done in a manner that accommodates the Sarepta Technology and Manufacturing processes, Catalent implements a quality management system acceptable to Sarepta, and Catalent maintains the [**] Facility ready for inspection by Sarepta or Regulatory Authority in connection with the BLA submitted by Sarepta. Catalent shall use commercially reasonable efforts to complete the validation of the [**] Facility in accordance with time frames agreed upon by Sarepta and Catalent under this Agreement and the Amended and Restated Collaboration Agreement. The [**] Facility will be designed in a manner that meets cGMPs, meets quality requirements acceptable to Sarepta and is in compliance with applicable Laws and Regulatory Acts. Catalent shall notify Sarepta in writing following completion of the construction and commissioning of the [**] Facility, and thereafter Sarepta shall audit the [**] Facility in accordance with the procedures set forth in this Agreement for purposes of supporting the Readiness Determination.

2.2. [**] Facility Design, Construction and Validation. Catalent may, at Catalent's discretion, design, construct and validate clean room suites at the [**] Facility, including purchasing and installing equipment suitable for manufacturing gene therapy products. The Parties may discuss in good faith the possibility of manufacturing one or more Sarepta products at the [**] Facility. Sarepta shall, at Catalent's request, reasonably cooperate with Catalent to assure that the design of the [**] Facility accommodates the Sarepta Technology and Manufacturing Processes. If the Parties agree that it would be desirable to Manufacture Sarepta product(s) at the [**] Facility, once the [**] Facility is constructed and validated the Parties will negotiate in good faith a plan to validate the Manufacture of one or more Sarepta products at the [**] Facility. Such plan will include allocations of costs and other responsibilities for the validation of the Manufacture of Sarepta products at the [**] Facility.

2.3. [**] Facility. Catalent operates the [**] Facility which currently has the capability to prepare working cell banks, develop products and processes, and fill and finish clinical and commercial drug products. Catalent may perform certain Manufacturing activities described herein at the [**] Facility, provided that (a) Sarepta is given prior notice and provides prior approval of such Manufacturing activities, and (b) such Manufacturing Activities are otherwise consistent with all requirements applicable to such Manufacturing activities under this Agreement, including, without limitation, compliance with the Quality Agreement and applicable Laws. Subject to the Parties' agreement, Manufacturing activities at the [**] Facility may be transferred to the [**] or [**] Facility.

2.4. Facility Licensure and Maintenance. Catalent will take all necessary actions to maintain in full force and effect at all times during the Term of this Agreement all permits, licenses, approvals and authorizations that allow Catalent to carry out its obligations under this Agreement, including any licenses in connection with Manufacturing in the Catalent Facility, and including, after approval of the Lead DMD Product, permits, licenses, approvals and authorizations in connection with licensed Manufacture in the Catalent Facility relating to the Bulk Drug Substance and Drug Product. Catalent shall operate and maintain the Catalent Facility and all equipment used in the Manufacture of Lead DMD Product in compliance with cGMPs, applicable Laws and Regulatory Acts, and the then current Quality Agreement between Sarepta and Catalent, and shall maintain a quality management system acceptable to Sarepta and assure the capacity of the Catalent Facility is sufficient to meet the requirements of this Agreement. Catalent shall not perform any Development, Manufacturing or Commercialization activities with respect to any Sarepta Drug Product at a facility other than the Catalent Facilities (as defined herein) without Sarepta's prior written consent.

2.5. Inconsistent Activities. During the use of the Sarepta Clean Room Suites for the Manufacture of Bulk Drug Substance pursuant to this Agreement, Catalent agrees that Catalent will not permit any activities within the Sarepta Clean Room Suites that are incompatible with and/or could compromise the Bulk Drug Substance being Manufactured by Catalent pursuant to this Agreement. Catalent shall be responsible, at its expense, to ensure that appropriate testing, cleaning, validation of cleaning procedures, remediation and other activities are undertaken in accordance with the Quality Agreement to provide for the readiness of the Catalent Facility for Manufacturing the Bulk Drug Substance or Drug Product [**], including to ensure no cross-contamination.

2.6. Employees. Catalent also shall be responsible for providing and/or hiring appropriately qualified personnel, including personnel with expertise in technical development, Manufacturing, operations, quality assurance, quality control and regulatory affairs, in accordance with a staffing plan agreed upon by Sarepta, such staff to be capable of operating the Catalent Facility in accordance with cGMPs and the Quality Agreement. Catalent shall assure that such staff receive appropriate training to carry out the responsibilities assigned to them.

2.7. No Obligation to Perform. Neither Catalent nor its Affiliates shall be obligated to Manufacture any Sarepta Drug Product for sale in any countries that are targeted by the comprehensive sanctions, restrictions or embargoes administered by the United Nations, European Union, United Kingdom, or the United States if it is prevented from doing so, or would be required

to obtain or apply for special permission to do so, due to any restriction (such as an embargo) imposed on it by any Governmental Authority, including those imposed by the U.S. Department of the Treasury's Offices of Foreign Assets Control.

ARTICLE III
SAREPTA MATERIALS

3.1. Sarepta Materials. Sarepta will provide to Catalent the Sarepta Materials for use in the Manufacture of Lead DMD Product in accordance with this Agreement. Catalent shall maintain a written record setting forth the type and name of all Sarepta Materials, the amount provided, the date provided, amounts used and the purpose of each use, and such other information as is appropriate to adequately record, track and account for all Sarepta Materials. [**].

3.2. Ownership and Return of Sarepta Materials. All Sarepta Materials shall remain the sole property of Sarepta, shall be used by Catalent only in carrying out its obligations under this Agreement and for no other purpose, shall not be transferred to any Third Party that is not specifically authorized in advance and in writing by Sarepta, and shall be returned to Sarepta upon request by Sarepta and at Sarepta's expense at the termination of this Agreement or when no longer being used, upon request by Sarepta and at Sarepta's expense.

3.3. Due Care. Catalent will use and store the Sarepta Materials with due care and in compliance with the applicable Storage Guidelines. Title and risk of loss or damage to such Sarepta Materials will at all times remain with [**], provided, however, that the foregoing shall not limit Catalent's liability for any damage to or loss of Sarepta Materials [**].

ARTICLE IV
TECHNOLOGY TRANSFER ACTIVITIES AND CLINICAL SUPPLY

4.1. Scope of Work. Upon the Original Effective Date of this Agreement, Catalent and Sarepta commenced with implementation of the Lead DMD Product Scope of Work attached hereto as **Exhibit B** (as the same may be amended or modified pursuant hereto, the "**Scope of Work**" or "**SOW**"). The Scope of Work is comprised of one or more of the following activities: (a) transfer of the Sarepta Technology, including [**], to Catalent to enable the performance of the other phases of the Scope of Work (the "**Technology Transfer Activities"**), (b) performance of certain Development activities, including process development and analytical testing work on the Lead DMD Product (the "**Development Activities**"), (c) using the Sarepta Technology Manufacturing process (including any Improvements thereto), performance of engineering runs and cGMP Manufacturing for Sarepta's clinical trials (the "**cGMP Manufacturing Runs**"), and (d) performance of process qualification in support of Sarepta's anticipated regulatory filings with the FDA ("**Process Performance Qualification**" or "**PPQ**"). With respect to each of the foregoing activities, Catalent shall submit for written approval by Sarepta that each phase is completed, and such approval shall not be unreasonably withheld or delayed. Additional activities may be included and made part of the Scope of Work, provided that any such additional activities are (1) included in a written supplemental exhibit that is sequentially numbered (*e.g.*, B-1, B-2, etc.), (2) specifically makes reference to this Agreement, (3) incorporates the terms and conditions hereof

by reference, and (4) includes a detailed description of the additional activities to be performed, the pricing, any equipment to be procured by Catalent at Sarepta's expense, including procurement and other related costs, and a signature block for both Parties. Once executed by the Parties, each additional exhibit shall be deemed part of, subject to and governed by the Agreement. In the event of any conflict between the Scope of Work and this Agreement, the terms of this Agreement shall prevail.

4.2. Technology Transfer Activities and Development Activities.

(a) Catalent and Sarepta shall each diligently perform the Technology Transfer Activities and the Development Activities in a professional and workman-like manner and in accordance with the activities, timeline and budget set forth in the Scope of Work. Catalent shall commit to the Technology Transfer Activities and the Development Activities appropriately qualified personnel, including, as appropriate, personnel with expertise in Development, Manufacturing, operations, quality assurance, quality control, and regulatory affairs. Sarepta shall provide reasonable assistance in connection with the Technology Transfer Activities and Development Activities and shall oversee the Technology Transfer Activities and the Development Activities, including [**]. Each Party shall comply with any applicable regulatory requirements in connection with the performance of the Technology Transfer Activities and the Development Activities.

(b) The Parties acknowledge that it is unlikely that this Agreement will cover each and every item and action that will be needed for the Technology Transfer Activities and the Development Activities. Instead, this Agreement is meant to establish an appropriate business relationship and basis upon which the Parties can continue to cooperate in good faith from time to time as additional transitional issues are identified after the Effective Date. For the avoidance of doubt, it is intended that the Parties shall use their respective good faith efforts based on their responsibilities to facilitate the timely progress of the Technology Transfer Activities and the Development Activities.

4.3. cGMP Manufacturing Runs. Following the completion of the Technology Transfer Activities and the Readiness Determination of Sarepta Clean Room Suites 3 and 4, and pursuant to a schedule agreed upon by the JSC, Catalent will commence with the performance of the tasks associated with the cGMP Manufacturing Runs. These tasks include [**] in accordance with cGMPs, the Specifications, the Quality Agreement, any applicable Laws and otherwise in accordance with this Agreement. Drug Product for [**] will be Manufactured at the Catalent Facility, as determined by Catalent and approved in writing by Sarepta, which approval shall not be unreasonably withheld, and in accordance with cGMPs, the Specifications, the Quality Agreement, any applicable Laws and otherwise in accordance with this Agreement. Catalent agrees to use commercially reasonable efforts to meet the Delivery timelines that have been established [**]. Catalent shall also perform the following relating to the Manufacture of the Lead DMD Product Bulk Drug Substance and Drug Product, as more fully set forth in the Scope of Work, the Specifications and the Quality Agreement:

(a) **Testing, Characterization and Release**. Catalent shall perform testing, characterization and release of Batches of Bulk Drug Substance and/or Drug Product as provided

in the Quality Agreement. The responsibility for the testing of certain Raw Materials will be set forth in the Quality Agreement.

(b) **Stability Testing**. Catalent shall perform stability testing and issue results in accordance with the Quality Agreement.

(c) **Storage**. Catalent shall perform all storage of Bulk Drug Substance and Drug Product in accordance with the Storage Guidelines.

4.4. Process Performance Qualification. Catalent shall perform Process Performance Qualification in support of Sarepta's BLA submission to the FDA for the Lead DMD Product in accordance with cGMPs, the Specifications, the Quality Agreement, any applicable Laws, and otherwise in accordance with this Agreement. Such Process Performance Qualification includes, [**]. Catalent agrees to use commercially reasonable efforts to meet the timelines set forth in the Scope of Work. The Process Performance Qualification will be completed upon Catalent's completion of the number of lots of Bulk Drug Substance and Drug Product specified in the Scope of Work, as the same may be amended as provided herein, that each conform to the Product Requirements (collectively, the "**PPQ Lots**"), along with corresponding documentation, Certificate of Analysis, Certificate of Compliance and validation reports for each such lot. The date upon which a final report confirming the completion of these PPQ activities has been submitted by Catalent and approved by Sarepta shall be referred to herein as the "**PPQ Completion Date**." Sarepta's approval of the completion of the PPQ activities shall not be unreasonably withheld or delayed.

4.5. Validation and Stability Studies.

(a) **General**. Catalent shall perform on an on-going basis all validation and stability studies required by, and in accordance with, the Specifications, cGMPs, applicable Laws and Regulatory Acts, and the Quality Agreement in connection with the Manufacturing of Bulk Drug Substance and Drug Product for the Lead DMD Product.

(b) **Duties**. Pursuant to a Scope of Work or Purchase Order, Catalent shall perform such validation and/or stability studies in accordance with the following:

(i) prepare and provide to Sarepta, in the format designated in the Quality Agreement, applicable Regulatory Materials as required for submission to any Regulatory Authority;

(ii) pull, store and analyze data and maintain, for periods of time set forth in the Quality Agreement, a database containing applicable Regulatory Materials, and provide Sarepta and any applicable Regulatory Authorities with such cooperation as is requested to respond to regulatory inquiries or investigations;

(iii) notify Sarepta promptly and in accordance with the timing set forth in the Quality Agreement if any Batch of Bulk Drug Substance or Drug Product fails any stability test(s), and initiate an investigation of such failure, promptly provide to Sarepta

all test results from the investigation and consult with and reach agreement with Sarepta on corrective actions to be taken; and

(iv) report to Sarepta promptly and in accordance with the timing set forth in the Quality Agreement any atypical results, deviations or adverse trends exhibited during testing, or process deviations, including, by way of example only, those which might reasonably be expected to impact quality or physical characteristics of the Bulk Drug Substance and/or Drug Product, and initiate an investigation thereof, promptly providing to Sarepta all test results from the investigation, and consulting with and reach agreement with Sarepta on corrective actions to be taken in accordance with the Quality Agreement.

4.6. Shipment; Title/Risk of Loss.

(a) **Acceptance of Batch Documentation**. In the case of Batches designated as cGMP Batches per the Scope of Work or Purchase Orders issued prior to Lead DMD Product Launch, Catalent will Manufacture them in accordance with the Product Requirements and Catalent will store and ship them in accordance with the applicable provisions of the Quality Agreement and the Storage Guidelines. As soon as Catalent has determined that a Batch complies with the Product Requirements, Catalent will send to Sarepta by a mutually agreed upon method the Batch Documentation. Sarepta will review the Batch Documentation to confirm that it meets the Product Requirements and to determine the appropriate use of that Batch. Within [**] of receipt of Batch Documentation, unless otherwise agreed to by the Parties, Sarepta shall either provide its acceptance of the Batch Documentation in accordance with the procedures set forth in the Quality Agreement ("**Sarepta Approval**") or, in the event Sarepta has identified specific issues or deviations with the Batch Documentation, give written notice thereof to Catalent, and such specific issues or deviations shall be resolved by the Parties in accordance with the Quality Agreement. [**] Upon Sarepta Approval, the Batch Documentation will be deemed approved and the relevant Batch of Product may be made available for shipment as provided in Section 4.6(b), provided that the granting of Sarepta Approval with respect to a Batch shall not preclude a subsequent claim with respect to any Latent Defect in such Batch in accordance with Section 8.3.

(b) **Shipment.** Following Sarepta Approval, and when Sarepta requests shipment of Batches of Bulk Drug Substance or Drug Product, Catalent will deliver such Batches to Sarepta, or to a location designated by Sarepta, including any Catalent or CPS facilities where additional Services are to be performed, as requested, for shipment from the Catalent Facility [**]. The term "**Delivery**" of Batches of Bulk Drug Substance or Drug Product shall have the same meaning as that set forth in Section 5.9(b). Catalent will store Bulk Drug Substance in accordance with the Quality Agreement and the Storage Guidelines as provided in Section 5.9(b).

(c) **Title/Risk of Loss.** Title to and risk of loss with respect to any Batch of Bulk Drug Substance and any Batch of Drug Product resulting therefrom shall pass [**].

(d) **Delivery Timing.** The timing of Delivery of clinical supply of Bulk Drug Substance and Drug Product Manufactured prior to the Lead DMD Product Launch will be as set forth in the Scope of Work, the applicable Purchase Order or otherwise agreed to by the Parties.

4.7. Invoicing; Payments to Catalent.

(a) **Scope of Work**. As set forth in the Scope of Work attached hereto as **Exhibit B**, Sarepta and Catalent have agreed upon pricing for the Technology Transfer Activities, the Development Activities, cGMP Manufacturing Runs and Process Performance Qualification activities to be performed by Catalent under this Agreement. With respect to services performed by Catalent prior to Lead DMD Project Launch other than the Manufacture of Bulk Drug Substance and/or Drug Product pursuant to a Purchase Order, including but not limited to [**] will create, with input from [**], and the Parties shall mutually agree upon a customized schedule for payments (each, a "**Payment Schedule**") to be included in the Scope of Work. Upon the execution of any Change Order or a material shift in the anticipated timeline of the payment obligations, Catalent shall promptly provide Sarepta with an updated Payment Schedule reflecting the corresponding updates to the timeline and/or timing of payments set forth therein. Catalent shall provide invoices to Sarepta for payments as set forth in the applicable Payment Schedule. With respect to any amounts that are due upon the completion of any milestones or deliverables set forth in the Scope of Work, [**], in its reasonable discretion, will determine when such milestone or deliverable is complete. Each such payment shall be due and payable within [**] following the receipt of the invoice. If [**] proposes to adjust the amounts set forth in the Scope of Work or the corresponding Payment Schedule, the Parties shall follow the procedures set forth in Section 4.8 with respect to a Change Order, and no such adjustment shall take effect absent a Change Order with respect thereto.

(b) **Invoicing for Statements of Work**. Catalent will invoice Sarepta as specified in an applicable SOW. Each such payment shall be due and payable within [**] following the receipt of the invoice.

(c) **Pass-Through Costs**.

(i) Catalent shall provide monthly invoices to Sarepta for all Pass-Through Costs arising under a Scope of Work, including [**], as applicable, provided that, (i) in the event that purchases are projected to exceed [**]. Notwithstanding anything to the contrary in this Agreement, payment for amounts invoiced by Catalent shall be due and payable within [**] following receipt of any other invoice. Raw Materials for Batches ordered under Section 5.3 will be invoiced in accordance with Section 5.8.

(ii) At least [**] prior to the initiation of each major activity of the Scope of Work, Catalent will prepare and provide to Sarepta a good-faith itemized estimate (an "**Estimate**") of expected costs and expenses to be incurred by Catalent for Raw Materials and any outsourced services which the Parties mutually agree to treat as a Pass-Through Cost. If the actual cost associated with a major activity will exceed the Estimate by more than [**], Catalent shall notify Sarepta [**].

(d) All payments to Catalent by Sarepta shall be in United States currency and shall be by wire transfer to:

[**]

(e) Notwithstanding anything to the contrary herein, all invoices issued pursuant to this Agreement shall be payable within the time periods expressly set forth herein unless Sarepta notifies Catalent of a disputed invoice amount. Sarepta will pay all undisputed amounts of such invoice within the applicable payment term period. In the case of a disputed amount, the Parties will in good faith discuss the item and seek resolution. Any unresolved dispute pertaining to an invoice amount or the payment obligation with respect thereto shall be resolved in accordance with Article XX of this Agreement, provided that any dispute relating to quality matters (e.g., compliance with cGMP and conformity of the Lead DMD Product with the Product Requirements) shall be raised and resolved in accordance with the Quality Agreement. Sarepta will pay all undisputed amounts of such invoice, if any, within the applicable payment term period. In the event that Sarepta has not paid any undisputed invoice amounts on or before the applicable due date, such failure shall be considered a material breach under this Agreement, subject to applicable cure provisions. [**]

4.8. Change Orders

(a) **Catalent Initiated Changes.** Before Catalent may amend or change the Scope of Work, Catalent shall prepare a Change Order describing in reasonable detail the nature of such change(s) and propose such Change Order to Sarepta for Sarepta's review and written approval. All approved Change Orders shall be approved and signed by the Project Manager of each Party or by such other authorized representatives of Catalent and Sarepta that the Project Managers may designate. If any changes contemplated by a Change Order will have a financial or other impact on the Scope of Work, Catalent shall include in the Change Order a written description of such impacts. If the authorized Sarepta Representative approves the Change Order notwithstanding Catalent's notice of any resulting cost increase, Sarepta shall reimburse Catalent for the cost of such changes as detailed in the Change Order. Upon approval of the Change Order, the Change Order will be implemented [**] in a good faith effort to meet the development and manufacturing timelines as set forth in the Scope of Work.

(b) **Sarepta Requested Changes.** Sarepta shall have the right to request reasonable modifications to the Scope of Work by providing notice thereof to Catalent. Upon receipt of such notice, Catalent shall generate a Change Order in accordance with the process described in Section 4.8(a) and submit such Change Order to the Sarepta authorized representative for review and approval. If the Sarepta authorized Representative approves the Change Order notwithstanding Catalent's notice of any resulting cost increase, Sarepta shall reimburse Catalent for the cost of such changes as detailed in the Change Order. Upon approval of such Change Order by the authorized Representative of each Party, the Change Order will be implemented [**] in a good faith effort to meet the development and manufacturing timelines as set forth in the Scope of Work or as described in the Change Order.

(c) The procedures of Sections 4.8(a) or (b) shall be employed for any changes requested to document(s) which are part of Catalent's cGMP document system provided that the approval of the appropriate Catalent department heads (including the Quality Assurance department) shall be required prior to any such change and any such changes are in compliance with cGMPs, the Specifications, the Quality Agreement, any applicable Laws and otherwise in

accordance with this Agreement. In the event of any requested change to a Sarepta-specific document, Sarepta approval shall also be required.

4.9. Other Related Services. Catalent shall provide such other services relating to the Lead DMD Product as agreed to between the Parties in a scope of work, which shall include the scope and fees for any such services and the terms and conditions of this Agreement shall govern and apply to such services.

ARTICLE V
MANUFACTURE AND COMMERCIAL SUPPLY OF LEAD DMD PRODUCT

5.1. General Requirements. Sarepta is developing the Lead DMD Product pursuant to a clinical development plan that currently projects a potential Marketing Authorization in 2023. Pursuant to the terms and conditions of this Agreement, and upon the receipt of a Purchase Order from Sarepta under this Agreement, Catalent agrees to Manufacture commercial supply of Lead DMD Product Bulk Drug Substance and Drug Product at the Catalent Facility.

5.2. Purchase of Lead DMD Product.

(a) Upon receipt of a Purchase Order from Sarepta submitted in accordance with Section 5.3, Catalent will Manufacture the Lead DMD Product in accordance with the Product Requirements, cGMPs, the then-current Quality Agreement and any applicable Laws and otherwise in accordance with this Agreement.

(b) In connection with the Manufacture of a Batch ordered in accordance with Section 5.3, Catalent shall charge Sarepta the Batch Price set forth in Section 5.5, as applicable. Upon commencement of the Post-Launch Period, Sarepta will order at least[**] of its annual commercial requirements for the Bulk Drug Substance and Drug Product (the "**Minimum Order Quantity**") from Catalent. Sarepta may maintain dedicated Sarepta Clean Room Suites [**] by placing [**] (the "**Minimum Annual Threshold**"). Sarepta may satisfy the Minimum Annual Threshold with orders for any services or products manufactured using [**].

(c) [**] is established pursuant to the Amended and Restated Collaboration Agreement and shall be reviewed on a periodic basis by the JSC (or a subcommittee thereof designated by the JSC) and may change from time to time based upon the final manufacturing process and/or changes implemented as a result of the Continuous Improvement Program.

(d) Provided that Catalent is in compliance with the terms of this Agreement, prior to Sarepta's engagement of a Third Party [**] to Manufacture Bulk Drug Substance or Drug Product of the Lead DMD Product, Sarepta shall provide Catalent written notice and the opportunity to meet the additional supply needs. Sarepta and Catalent shall promptly meet to discuss the details of the additional supply needs including, [**] important details. [**]

5.3. Purchase Orders; Forecasts; Procedures.

(a) Upon receipt of a Purchase Order from Sarepta pursuant to this Agreement, Catalent shall Manufacture Batches of Bulk Drug Substance and/or Drug Product in accordance with the Product Requirements, cGMPs, the then-current Quality Agreement and any applicable Laws and otherwise in accordance with this Agreement. [**]

(b) With respect to orders of Batches of Bulk Drug Substance, Sarepta shall provide forecasts of its ordering needs and, as set forth in Section 5.3(f) below, place with Catalent Purchase Orders for the supply of Bulk Drug Substance of Sarepta Drug Products being Manufactured by Catalent pursuant to this Agreement. Such Purchase Orders shall also include orders for Drug Product as needed.

(c) **Initial Forecast**.

(i) The Parties have agreed to an initial forecast (the "**Initial Forecast**") through [**].

(ii) Subject to the Initial Forecast Refresh (defined below) and the rights to make other adjustments set forth herein, the Initial Forecast will be binding as to [**] (such quarters are each a "**Binding Quarter**" or "**BQ**" and the entire period is the "**Binding Orders Period**"). [**] (the "**Initial Forecast Refresh**"), [**]. The Initial Forecasts and subsequent Rolling Forecasts shall be provided by Sarepta to Catalent in a form of notice substantially similar to the example notice provided in **Exhibit D**.

(d) **Rolling Forecast**. Prior to [**], Sarepta will refresh its forecast and extend the forecast to [**]. In the rolling forecast, the [**] shall be BQs, and the [**] of the rolling forecast shall be non-binding (each such quarter a "**Non-Binding Quarter**" or "**NBQ**"). When the rolling forecast is refreshed, the previous [**] is added to the forecast (such forecasting then becomes the "**Rolling Forecast**").

(e) **Forecast Modification; Monitoring**. In addition to the Initial Forecast Refresh, Sarepta will be afforded the flexibility to make modification requests to the Initial and Rolling Forecasts, as set forth below (each a "**Forecast Modification**"). Subject to the Minimum Annual Threshold, if a Forecast Modification is made for [**] that requests Manufacture of a Batch of Sarepta drug product other than that which was originally forecasted, Catalent shall use commercially reasonable efforts, but shall not be obligated, to Manufacture the Batch of different Sarepta drug product; and if such a Forecast Modification is made with respect to [**], Catalent shall be obligated to Deliver such Batches of different Sarepta drug product in accordance with a Delivery schedule to be agreed by the Parties at the time of the Forecast Modification. If a Forecast Modification is made for [**] that requests additional Batches over what was originally forecast (the "**Additional Batches**"), Catalent shall use commercially reasonable efforts, but shall not be obligated, to Manufacture the Additional Batches. If a Forecast Modification is made for [**] that requests Additional Batches (subject to the Maximum Annual Batches cap), as provided in Section 5.3(a), Catalent shall be obligated to Deliver such Batches in accordance with a Delivery schedule to be agreed by the Parties at the time of the Forecast Modification. If the Forecast Modification results in a reduction of Batches to be Delivered by Catalent during the Binding Orders Period, Sarepta shall be obligated to pay Catalent for all Batches ordered for [**]. During the period in

which Catalent is Manufacturing Bulk Drug Substance and/or Drug Product, the JSC will have periodic teleconferences or meetings to monitor and review the status of Manufacturing operations and to address any issues that may arise.

(f) **Production Schedule; Purchase Orders; Delivery Dates**. Within [**] following Catalent's receipt of the Initial Forecast and each Rolling Forecast thereafter, Catalent shall prepare, with input from Sarepta, a Batch production schedule for the Binding Orders Period which includes the order of production (including anticipated start dates) for each Batch of Bulk Drug Substance and Drug Product forecasted and the anticipated delivery to Sarepta of the Batch Documentation for each such Batch (the "**Production Schedule**"). In preparing the Production Schedule, the production timeline for each Batch of Bulk Drug Substance or Drug Product being Manufactured during the Binding Orders Period shall be based upon reasonable time estimates for the Manufacture/Release Period, delivery of Batch Documentation, Sarepta's review and approval of the Batch Documentation and a reasonable amount of time for the investigation and clearance of deviations that may have occurred during the Manufacture/Release Period. Within [**] from Sarepta's receipt of the Production Schedule, Sarepta shall submit to Catalent binding Purchase Orders specifying the number of Batches, the Sarepta Drug Product or Bulk Drug Substance being Manufactured, and the anticipated Delivery dates as specified in the Production Schedule. If Catalent is unable to Deliver the required Batches specified in a Purchase Order on or before the anticipated Delivery date set forth in the Production Schedule, Catalent shall notify Sarepta promptly upon discovery of its inability to meet the Production Schedule. [**]. All acknowledged Purchase Orders submitted pursuant to this Agreement shall be binding on Catalent and Sarepta. No later than [**] after Sarepta's submission of a Purchase Order, Catalent shall provide Sarepta with written acknowledgment of receipt of the Purchase Order. Catalent shall timely Manufacture and Deliver the amounts ordered by Sarepta in accordance with Production Schedule, and Catalent shall use commercially reasonably efforts to Manufacture and Deliver any Additional Batches; it being understood that Catalent's failure to supply such Additional Batches shall not constitute a breach under this Agreement.

(g) **Delays and Suspensions**. The provisions in this Section 5.3 are intended to apply to situations involving the ongoing, uninterrupted Manufacture of Sarepta Drug Products once the Lead DMD Product Launch has occurred. [**]

5.4. Key Performance Indicators. Sarepta and Catalent shall use Key Performance Indicators to evaluate Catalent's performance of its obligations under this Agreement. [**] prior to initiating Manufacturing activities under this Agreement, a list of the indicators that shall be the "**Key Performance Indicators"** shall be mutually agreed in writing by the Parties. [**] For the avoidance of doubt, none of the Key Performance Indicators will pertain to quality matters or quality attributes of the Lead DMD Product, which are governed by the Quality Agreement.

5.5. Batch Price. All Batches Manufactured pursuant to a Purchase Order issued pursuant to Section 5.3 will be charged on a per Batch basis (the "**Batch Price**").

(a) **Bulk Drug Substance**. For Batch orders placed pursuant to Section 5.3 [**], the Batch Price for Bulk Drug Substance shall be [**]. [**] The deliverables for a Bulk Drug Substance Batch include [**]. Upon completion of [**], and annually thereafter, the JSC will

evaluate and, if appropriate, recommend reasonable modifications to the Batch Price for Bulk Drug Substance that are reflective of and consistent with [**].

(b) **Drug Product**. The Batch Price for Drug Product shall be [**]. The deliverables for a Drug Product Batch include [**]. Upon completion of [**], and annually thereafter, the JSC will evaluate and, if appropriate, recommend reasonable modifications to the Batch Price for Drug Product that are reflective of and consistent with [**].

(c) All Raw Materials (other than Sarepta Materials) will be charged to Sarepta [**]. Catalent shall provide details of such Raw Materials on a recurring basis based on a mutually agreed upon reporting format. Estimates should generally be provided on an [**] basis as may be agreed between parties. Annually, in connection with the JSC's review of the Batch Price pursuant to this Section 5.5, the Parties agree to review the inventory of Raw Materials necessary to support the Batches of Bulk Drug Substance ordered by Sarepta for the Binding Orders Period, and the ordering and storage necessary to support the same. Such review shall be part of and subject to the Continuous Improvement Program set forth in Article X below.

(d) Pass-Through Costs incurred by Catalent will be charged to Sarepta at [**]. [**] the Parties will mutually agree upon a list of costs that are directly associated with the Manufacturing of Batches, including a reasonable estimate of annual costs supported by reasonable documentation.

(e) Starting in [**], Catalent may increase the Batch Price [**] ("**FY**") in accordance with increases in [**]. If the relevant [**] is not reported for January or December of the prior year, any increase or decrease shall be measured against the most recent year for which data is available for January and December.

(f) Catalent shall work with Sarepta to put in place a mutually accepted cost tracking system that efficiently and expeditiously provides the Parties with cost transparency for the procurement of the Raw Materials, including with respect to progress made in the Continuous Improvement Program.

5.6. Reserved.

5.7. Reserved.

5.8. Procurement. Catalent shall procure all Raw Materials (other than Sarepta Materials) necessary for the Manufacture of Batches of Bulk Drug Substance and Drug Product consistent with the then applicable Initial Forecast and Rolling Forecast. Such Raw Materials shall be used by Catalent in undertaking the Manufacturing activities contemplated by this Agreement for the benefit of Sarepta. Catalent will use and store the Raw Materials with due care and in compliance with the applicable Storage Guidelines. In connection with the establishment of the final manufacturing process, Catalent and Sarepta shall agree upon a bill of materials for each Batch of Lead DMD Product to be Manufactured by Catalent ("**BOM**") and Catalent shall provide to Sarepta an estimated cost of all of the items on the BOM (the **"Batch Materials Cost**"). The Batch Materials Cost may be updated [**] Catalent shall invoice Sarepta for [**] (each such

deposit, a "**Deposit**", and each such invoice for a Deposit, a "**Deposit Invoice**"). The amount of the Deposit shall be equal to [**]. [**] Notwithstanding anything to the contrary in this Agreement, Sarepta shall pay each Deposit Invoice within [**] of receipt of such Deposit Invoice. When Catalent invoices Sarepta upon Sarepta Approval of a Batch in accordance with Section 5.10, such invoice shall include [**]. If materials previously listed on the BOM are removed from the BOM (such materials, "**Non-BOM Materials**"), Catalent shall invoice Sarepta as soon as there is a change in BOM for Non-BOM Materials costs reasonably incurred by Catalent in reliance on the forecast for Non-BOM Materials [**]. Sarepta shall pay each non-BOM invoice within [**] of receipt of such invoice. [**]

5.9. Shipment; Title; Delivery.

(a) **Acceptance of Batch Documentation**. Following [**], Catalent will Manufacture all Batches of Lead DMD Product Bulk Drug Substance and Drug Product in accordance with the Product Requirements and Catalent will store and ship them in accordance with the applicable provisions of the Quality Agreement and the Storage Guidelines. As soon as Catalent has determined that a Batch complies with the Product Requirements, Catalent will send to Sarepta by a mutually agreed upon method the Batch Documentation. Sarepta will review the Batch Documentation to confirm that it meets the Product Requirements[**]. Within [**] of receipt of Batch Documentation, unless otherwise agreed to by the Parties, Sarepta shall either provide its Sarepta Approval or, in the event Sarepta has identified specific issues or deviations with the Batch Documentation, give written notice thereof to Catalent, and such specific issues or deviations shall be resolved by the Parties in accordance with the Quality Agreement. If Sarepta fails to respond within the foregoing time period with its acceptance of the Batch or provide written notice of issues or deviations with the Batch Documentation, Sarepta Approval of the Batch will be deemed to have occurred. Upon Sarepta Approval, the Batch Documentation will be deemed approved and the relevant Batch of Bulk Drug Substance or Drug Product may be made available for shipment as provided in Section 5.9(b), provided that the granting of Sarepta Approval with respect to a Batch shall not preclude a subsequent claim with respect to any Latent Defect in such Batch in accordance with Section 8.3.

(b) **General**. Following Sarepta Approval, and when Sarepta requests shipment of Batches of Bulk Drug Substance or Drug Product, Catalent will deliver such Batches to Sarepta, or to a location designated by Sarepta, including any Catalent [**] facilities where additional Services are to be performed, as requested, for shipment from the Catalent Facility [**]. For purposes of this Agreement, "**Delivery**" of a Batch of Bulk Drug Substance shall be deemed to have occurred upon [**]. Also for purposes of this Agreement, Delivery of a Batch of Drug Product shall be deemed to have occurred upon [**]. Catalent will store Bulk Drug Substance, at no [**], until the earlier of (a) the date on which such Bulk Drug Substance is utilized in the manufacture of Drug Product hereunder and (b) [**] following Sarepta Approval thereof; and Catalent will store Drug Product, [**] for up to [**] following Sarepta Approval thereof. Sarepta shall be responsible for Storage Costs for storage beyond the foregoing time periods. All storage of Bulk Drug Substance and Drug Product shall be performed in accordance with the Storage Guidelines and the Quality Agreement. [**]

(c) **Title/Risk of Loss**. Title to and risk of loss with respect to any Batch of Bulk Drug Substance and any Batch of Drug Product resulting therefrom shall pass from [**] to [**] upon the Delivery of such Batch of Bulk Drug Substance, [**].

5.10. Invoices and Payment.

(a) **Invoices for Batches Delivered**. Catalent may invoice Sarepta for Batches of Bulk Drug Substance and Batches of Drug Product ordered by Sarepta on or after[**]. Following [**], Sarepta shall pay all undisputed invoices of Catalent for such Batch within [**] after the date that Sarepta receives an invoice from Catalent. Any dispute of the invoiced amount or the payment obligation with respect thereto shall be raised and resolved in accordance with Section 4.7(e) of this Agreement, provided that any dispute relating to quality matters (e.g., compliance with cGMP and conformity of the Lead DMD Product with the Product Requirements) shall be raised and resolved in accordance with the Quality Agreement. All invoices shall be submitted to the address designated from time to time in writing by Sarepta. Notwithstanding anything to the contrary in this Agreement, if at any time any payment is not received by Catalent by its due date, then Catalent may, in addition to other remedies available at law or in equity, [**] until paid in full (or, if less, the maximum amount permitted by the governing law), in each case without releasing Client from its obligations under this Agreement. [**] All payments to Catalent by Sarepta shall be in accordance with Section 4.7(d). All invoices shall include, at a minimum, the following:

(i) the quantity of Batches, the lot numbers associated with each Batch, and the corresponding Sarepta Purchase Order;

(ii) the charges associated with such Batches, which shall be made according to the Batch Price set forth in this Agreement;

(iii) Unless invoiced separately, Batch-related outsourcing, shipping and other non-Facility related direct costs incurred by Catalent [**], as described in Section 5.5(d) [**], all subject to reconciliation under Section 5.8; and

(iv) such other information as Sarepta may reasonably request from time to time.

(b) **Minimum Annual Threshold.** On the last Business Day of each calendar quarter and in accordance with Section 5.2, Catalent shall determine the balance [**] owing to Catalent with respect to the Sarepta Clean Room Suites. To the extent a balance is determined to be owed, Catalent shall provide to Sarepta a statement of the amount owing, which shall include a summary report on the nature and amount of any credits applied, so that Sarepta may promptly review and verify the foregoing. Following verification of the statement[**], which invoice shall be paid within [**] from the date the invoice is received by Sarepta.

5.11. Compliance.

(a) **General**. Catalent shall (i) Manufacture, test, package, store, label, release and Deliver Batches of Bulk Drug Substance and Drug Product and (ii) maintain the Catalent

Facility, all storage facilities and all stored Raw Materials, in each case, in accordance with Specifications, cGMPs, applicable Laws and Regulatory Acts and the Quality Agreement.

(b) **Compliance Throughout the Term with Specifications, cGMPs, Applicable Laws and Regulatory Acts and the Quality Agreement**. Catalent shall be responsible for identifying and implementing any actions required to bring Catalent into compliance with Specifications, cGMPs, applicable Laws and Regulatory Acts and the Quality Agreement throughout the Term of this Agreement. Catalent shall implement any such changes as promptly as practicable after the changes are adopted (even if, in the case of cGMPs and Regulatory Acts, a later effective date is specified), unless the effective date falls after a termination of this Agreement for which notice has been previously given. [**]

5.12. Specification Change; Change Orders.

(a) **Catalent Initiated Changes**. Catalent shall not make any revisions to the Specifications or to the manner of storing and handling materials used in the Manufacture of the Bulk Drug Substance or Drug Product or change the supplier(s) of Raw Materials, without prior written consent of Sarepta in accordance with the Quality Agreement. Sarepta retains the right and responsibility for final approval of the Specifications for Bulk Drug Substance and Drug Product, including in connection therewith the right to approve any changes in the manner of storing and handling materials and in the supplier(s) of Raw Materials. All requests by Catalent for such revisions shall be submitted in writing to Sarepta on the forms prescribed by the Quality Agreement in advance. Catalent shall notify Sarepta, in writing and in reasonable detail, of: (i) Catalent's suggested change; (ii) the reasons for the suggested change; (iii) the perceived benefits of the suggested change to Catalent and Sarepta, respectively; and (iv) the estimated costs and timing of implementing such change. If the Parties implement a change in the Specifications hereunder, they shall negotiate any changes in any affected Purchase Order to provide reasonable accommodation for changed circumstances. Catalent shall be responsible for documenting all revisions to the Specifications, subject to Sarepta's approval, in accordance with the Quality Agreement, as applicable. Catalent shall also support all applicable regulatory submissions in connection therewith and, at the request of Sarepta, address any questions or inquiries related thereto.

(b) **Sarepta Requested Changes**. Sarepta shall be entitled to change Manufacturing parameters and release for Bulk Drug Substance or Drug Product from time to time in its discretion, or to change any Specifications at the request, or as required, by any Governmental Authority and, provided that the Sarepta Requested Change(s) does not [**], Catalent shall make all revisions to the Specifications requested by Sarepta as described in the Quality Agreement and develop, support and submit any Catalent Facility-related regulatory submissions in a timely fashion to support Sarepta's effecting that change. Sarepta retains the right and responsibility for final approval of Specifications for Bulk Drug Substance and Drug Product. Sarepta shall pay Catalent the documented amounts incurred in implementing a change to the Specifications requested by Sarepta under this Section 5.12(b). [**]

5.13. Validation and Stability Studies.

(a) **General**. As mutually agreed upon between the Parties, Catalent shall perform on an on-going basis all validation and stability studies required by, and in accordance with, Specifications, cGMPs, applicable Laws and Regulatory Acts and the Quality Agreement in connection with the regular course of Manufacturing Bulk Drug Substance and Drug Product for supply hereunder.

(b) **Duties**. In performing its duties under Section 5.13(a), Catalent shall perform the following tasks:

(i) prepare and provide to Sarepta, in the format and level of detail designated by Sarepta, the data package for required regulatory submissions;

(ii) pull, store and analyze data and maintain, for periods of time set forth in the Quality Agreement, database containing applicable information, and provide Sarepta and any applicable Regulatory Authorities with such cooperation as is requested to respond to regulatory inquiries or investigations;

(iii) notify Sarepta promptly, and in accordance with the timing set forth in the Quality Agreement, if any Batch of Bulk Drug Substance or Drug Product fails any stability tests; and

(iv) report to Sarepta promptly, and in accordance with the timing set forth in the Quality Agreement, any atypical results, deviations or adverse trends exhibited during Manufacture and/or testing.

5.14. Filling and Finishing. Catalent shall be responsible for filling and finishing of the Lead DMD Product, which shall be performed in accordance with the Specifications, cGMPs, applicable Laws and Regulatory Acts, and the Quality Agreement.

5.15. Technology Transfer. Notwithstanding anything to the contrary, upon [**] written notice to Catalent, Sarepta shall be permitted to transfer the Manufacturing process for the Lead DMD Product and any portion thereof, for the sole and limited purpose of the [**] to itself; its Affiliates; its and their [**] its and their [**] and [**] (such parties referred to collectively in this Section as the "**Transferees**").

(a) **Technology Transfer License**. In furtherance of the Lead DMD Product Technology Transfer, [**] ("**Technology Transfer License**"), provided that any such Transferee is subject to the obligations of confidentiality with respect thereto at least as restrictive as those contained herein.

(b) **Document Transfer**. In furtherance of the Lead DMD Product Technology Transfer, Catalent agrees to furnish to the Transferees all documents and information reasonably necessary to enable the practice of the Manufacturing processes for the Lead DMD Product. Such documents and information shall include, but are not limited to, [**].

(c) **Technology Transfer Fee.** For any transfer hereunder, Sarepta shall pay or obligate a Transferee to pay to Catalent a reasonable technology transfer fee (the "**Technology**

Transfer Fee") and all expenses associated therewith. Prior to the transfer, Catalent shall provide Sarepta a proposed Technology Transfer Fee based upon its reasonable assessment of the amount of time required to successfully effect the transfer to the transferee. Sarepta or another Transferee shall pay the Technology Transfer Fee as set forth in the proposal from Catalent.

ARTICLE VI
QUALITY ASSURANCE; QUALITY CONTROL

6.1. Quality Agreement. Promptly following the execution of this Agreement, and in any event no later than commencement of cGMP Manufacturing Runs, the Parties will negotiate and enter into a Quality Agreement with respect to the Manufacture of the Lead DMD Product for use in [**] clinical trials, the provisions of which will be incorporated herein by reference thereto and deemed a material part of this Agreement. Further, [**] the Parties agree to negotiate in good faith a new Quality Agreement, which will amend and supersede the initial Quality Agreement, and which will specifically address and govern the Manufacture of Lead DMD Product for [**] clinical trials and commercial use. In the event of a conflict between the terms of the Quality Agreement and the terms of this Agreement as pertaining to any quality matters, the terms of [**] shall control.

6.2. Quality Assurance; Quality Control. Catalent shall implement and perform operating procedures and controls for: (a) sampling, stability and other testing of Sarepta Materials, Bulk Drug Substance and, if applicable, Drug Product, (b) for validation, documentation and release of Bulk Drug Substance and, if applicable, Drug Product, and (c) such other quality assurance and quality control procedures as required by Specifications, cGMPs, applicable Laws and Regulatory Acts and the Quality Agreement. Only Bulk Drug Substance and, if applicable, Drug Product complying with the Product Requirements may be released by Catalent. Catalent shall ensure that Catalent's capacity for quality assurance and quality control activities are commensurate with the Binding Orders Period of the Rolling Forecast.

6.3. Certificates of Analysis and Certificate of Compliance. Catalent shall supply to Sarepta upon reasonable request copies of its analysis and data supporting the Certificate of Analysis and/or Certificate of Compliance of each Batch of Bulk Drug Substance and Drug Product provided under this Agreement.

6.4. Quality Control Tests. Catalent shall ensure that the Parties adopt from time to time quality control tests and that representative samples of Bulk Drug Substance and Drug Product are taken, analyzed and retained in accordance with the Specifications, cGMPs, applicable Laws and Regulatory Acts and the Quality Agreement, using quality control methods provided by or agreed with Sarepta and validated by Catalent prior to the release of the Batches.

6.5. Testing and Reference Standards. Catalent shall ensure that testing methodology and testing reference standards comply with Specifications, cGMPs, applicable Laws and Regulatory Acts and the Quality Agreement. Catalent shall provide to Sarepta as reasonably requested [**] sufficient quantities of reference standards for Bulk Drug Substance and Drug Product so as to enable Sarepta to carry out and/or maintain the necessary testing capability to

comply with its regulatory obligations and the obligations set out in cGMPs and the Quality Agreement throughout the Term of this Agreement.

6.6. Process Controls and Tests. Catalent shall institute and maintain process controls during the Manufacture of Bulk Drug Substance and Drug Product in accordance with the Specifications, cGMPs, applicable Laws and Regulatory Acts and the Quality Agreement. Further, Catalent shall maintain full records of such tests which shall upon request be made available to Sarepta together with retained in-process samples, in the event of a complaint or query arising in respect of Bulk Drug Substance and Drug Product. Such records and samples shall be retained by Catalent for a period of [**] from the time they were made or taken or, alternatively, delivered to Sarepta.

6.7. Adverse Trends. Catalent must report any adverse trends to Sarepta that arise during normal or stability testing of Bulk Drug Substance and Drug Product, or in connection with trending for process, which might reasonably be expected to impact the quality or physical characteristics of Bulk Drug Substance or Drug Product.

ARTICLE VII
BATCH PROCESSING; SUPPLY INTERRUPTION AND DELAYED DELIVERY

7.1. Batch Processing.

(a) The Parties acknowledge that Manufacturing of Batches of Bulk Drug Substance and Drug Product can be unpredictable and, even when all variables are appropriately controlled, it is difficult to predict how a Batch will perform while it is being Manufactured (the "**Batch Processing**") and results often vary. As a result, it is anticipated that the Specifications (which are part of the Product Requirements) that will be established for the Lead DMD Product for the purpose of determining whether a Batch may be released will include certain product specifications with min-max ranges. Yet, even accounting for acceptable variability within these ranges as part of in-process or release testing, there will be Batches that are terminated or rejected due to the testing results falling outside the specified ranges. Reasons for this may include, but are not limited to, one or more of the following events that may occur during the Batch Processing (each, a "**Terminating Event**"):

(i) Equipment used to Manufacture the Batch malfunctions;

(ii) The Sarepta Materials or Raw Materials used during the Batch Processing fail or are defective [**];

(iii) The Sarepta Materials or Raw Materials change or differ from previous lots of materials purchased from the same vendor [**];

(iv) The Batch becomes contaminated during the Batch Processing;

(v) A Catalent manufacturing associate or operator makes a mistake in processing the Batch [**]; and

(vi) A Catalent manufacturing associate or operator commits an act or omission of gross negligence or intentional misconduct resulting in the loss of the Batch.

(b) For the purposes of this Agreement, a Terminating Event that leads to the termination or rejection of an [**], Bulk Drug Substance or Drug Product Batch, including an event or series of events that causes the Batch to fail one or more in-process and/or release testing specifications, shall be referred to as a "**Failed Batch**". A Failed Batch is always a Batch that is terminated or rejected prior to the submission of Batch Documentation to Sarepta. A "**Defective Batch**" (see Section 8.4 below) is a Batch that is rejected or otherwise determined to have a Defect after the Batch Documentation is provided to Sarepta.

(c) Each Failed Batch and Defective Batch will be the subject of an investigation to be performed in accordance with the Quality Agreement (each as "**Batch Investigation**"). [**] The Batch Investigation will endeavor to determine the root (or primary) cause of the Batch termination or rejection (the "**Root Cause**") and the corrective measures to be taken in the Manufacture of future Batches of Bulk Drug Substance or Drug Product, as appropriate, to minimize of chances of or prevent the same type of root cause from happening again. The JSC will be advised of the findings and outcome of the Batch Investigation.

(d) Within [**] of the execution of this Agreement, each Party shall identify representatives with sufficient technical knowledge of the Drug Product and related Manufacturing processes to collaborate to establish a framework (the "**Framework**") for determining [**] (the "**Target**"), including [**] (e.g., Catalent may at [**]. It is contemplated that the Target will be determined using data from Batches Manufactured [**].

7.2. Termination of a Batch. [**] shall have full authority, subject to compliance with the Quality Agreement and the proviso below, to make the decision to terminate a Batch before completion and may confer with [**] during the Batch Processing to determine whether a Batch that is otherwise outside agreed upon process parameters (based upon in-process testing) may ultimately be accepted [**]. Prior to terminating a Batch, [**] may elect to confer with [**] regarding acceptance of the Batch despite the fact that it is out of trend. If Sarepta agrees to accept the Batch after notice of a deviation from agreed upon process parameters, it may not later reject the Batch for that reason. The Parties agree that with respect to meeting the quality and safety specifications of a Batch, a Batch that does not meet the Specifications will be deemed a Failed Batch. As set forth in Section 8 below and following Sarepta Approval of a Batch and Delivery by Catalent, Sarepta may later reject the Batch and deem it a Defective Batch if it determines through its own release testing that the Batch does not meet the Product Requirements.

7.3. Remedies for Failed Batches and Defective Batches. Catalent shall Reprocess or re-Manufacture all Failed Batches and Defective Batches regardless of the "root cause" that led to the termination or rejection of the Batch. The remedies set forth in Section 8.6 shall apply to all Failed Batches and Defective Batches.

7.4. Supply Interruption.

(a) **Delay Period**. Subject to the terms and conditions of this Agreement, Catalent shall timely fulfill the Purchase Orders and supply Batches that meet the Product Requirements in such numbers as are set forth in the applicable Purchase Order for each BQ and pursuant to the production schedule as approved by both Parties ("**Approved Production Schedule**"). Catalent will provide Sarepta as much advance notice as possible if Catalent is unable, or anticipates that it will be unable, to timely supply Lead DMD Product that meets the Product Requirements in accordance with the Approved Production Schedule. In the event of such an interruption (or anticipated interruption) in supply, the Parties will negotiate in good faith reasonable adjustments to the Approved Production Schedule so as to maintain the aggregate supply commitment set forth in the [**] BQs of the Binding Orders Period in which the supply interruption is anticipated to or does occur [**].

(b) **Sarepta Rights and Remedies**. If, during any such Delay Period and notwithstanding anything to the contrary in this Agreement, Catalent fails to supply Batches that meet the Product Requirements in such number [**] of the total number of Batches originally ordered in the [**] applicable to the Delay Period, which failure results from any cause other than a failure by Sarepta to timely supply Sarepta Materials, a breach of this Agreement by Sarepta or a suspension of production requested by Sarepta for which Section 5.3(e) applies, Sarepta shall have the option to [**]. [**] In the event the Failed Batches or Defective Batches were caused by a failure beyond Catalent's reasonable control, the Parties will discuss in good faith a reasonable and equitable outcome.

(c) **Termination of Delay Period**. If a Delay Period commences (i.e., if Catalent is unable to timely supply Lead DMD Product that meets the Product Requirements in accordance with the amounts and the schedule set forth in the Rolling Forecast with respect to any BQ), such Delay Period will be terminated if and only if Catalent is able to make up the deficiency in the number of Batches Delivered during any of the BQs covered by the Delay Period. For purposes of illustration, [**].

ARTICLE VIII
BATCH TESTING AND SAREPTA REVIEW; DEFECTIVE BATCHES

8.1. Testing. Catalent or its approved subcontractor will perform all testing set forth in the Specifications or otherwise provided for in the Quality Agreement to determine conformity with the Product Requirements and Catalent will issue a Certificate of Analysis and Certificate of Conformance based on the results thereof. Sarepta may elect to conduct additional release testing, in its sole discretion, in which case Sarepta shall work with Catalent to promptly undertake such testing and shall report any adverse findings to Catalent. If such additional testing is not deemed necessary, then Sarepta shall inform Catalent. Any testing which Sarepta elects to perform shall not relieve Catalent from its obligations to comply with the terms of this Agreement, including its obligations with respect to any Batch that is determined to be a Defective Batch.

8.2. Sarepta Review.

(a) Pursuant to Section 4.6(a) or 5.9(a), as applicable, Catalent will deliver the Batch Documentation to Sarepta following [**] and Sarepta shall review and advise Catalent as to

its determination of whether the Batch meets the Product Requirements. Sarepta's obligation to pay the invoice for any Batch about which it notifies Catalent of a nonconformity will be tolled until such time as the Parties have made a determination as to the conformity of such Batch and, if Catalent is required to remanufacture or Reprocess the Batch [**] pursuant to Section 8.6, until such time as Catalent delivers a Batch that conforms to the Product Requirements.

(b) Upon actual physical receipt of each Batch by Sarepta or its designee ("**Actual Sarepta Receipt**"), Sarepta (or its designee) will:

(i) inspect the Batch and confirm that the quantity received by Sarepta (or its designee) matches the quantity set forth in the Batch Documentation, and make all the necessary reserves on the delivery receipt related to any shortage in the quantity; and

(ii) inform Catalent by email of any shortage identified through the conduct of the inspection pursuant to Section 8.2(b)(i) within [**] from the date of Actual Sarepta Receipt of such Batch.

8.3. Latent Defects. Any Sarepta Approval or other acceptance of any Batch of Bulk Drug Substance or Drug Product by Sarepta shall not preclude a subsequent claim with respect to any Latent Defect, provided that: (i) Sarepta notifies Catalent of such Latent Defect no later than [**] after the discovery of the Defect, and (ii) such Latent Defect is mutually agreed upon between the Parties or otherwise determined in accordance with the dispute resolution procedures set forth in the Quality Agreement. In such event, Sarepta shall have the rights set forth in Section 8.6 with respect to such Batch.

8.4. Defects; Quarantine.

(a) **Satisfaction of Product Requirements**. Catalent shall not release any Batch for shipment that does not conform to the Product Requirements, without the prior written approval of Sarepta, and Sarepta shall have the right to reject any Batch that fails to satisfy the Product Requirements.

(b) **Catalent Notifications**. Without limiting any of the foregoing in this Article VIII, Catalent shall notify Sarepta [**] upon becoming aware of any problem related to the Manufacture of Batches under this Agreement including:

(i) where any Batch may be affected by bacteriological or other contamination, significant chemical, physical or other change or deterioration or stability failures; and

(ii) where any Batch may not comply with the Product Requirements therefor.

(c) **Quarantine**. In the event of any actual or alleged Terminating Event or Defect in a Batch, Catalent shall quarantine and properly tag such Batch. Catalent shall promptly submit to Sarepta a report detailing the nature of the Terminating Event or Defect, including the investigation and testing done and Catalent's recommended disposition of the Batch.

(d) **Defects Discovered by Sarepta**. In the event Sarepta discovers any actual or alleged Defect in a Batch:

(i) Sarepta shall notify Catalent in writing;

(ii) the payment obligation in relation to any such Delivery shall be suspended forthwith pending resolution of the dispute;

(iii) the Parties shall immediately endeavor to agree whether or not the Batch in question complies with the requirements of this Agreement;

(iv) Catalent shall be entitled at all reasonable times to inspect and/or analyze the relevant Batch;

(v) As set forth in Section 7.1(c), Catalent will conduct a Batch Investigation into each Defect in a Batch and provide any additional information regarding the Defect as may be reasonably requested by Sarepta; and

(vi) The ultimate disposition of a Batch determined to have a Defect will be the responsibility of [**] quality assurance department.

8.5. Resolution of Disputes Regarding Nonconformity or Defects. In case of any disagreement between the Parties as to whether a Batch contains a Defect, or as to the cause of any Terminating Event or Defect, the quality assurance representatives of the Parties will attempt in good faith to resolve any such disagreement and each Party will follow the dispute resolution procedures set forth in the Quality Agreement to determine whether such product contains a Defect and/or the cause of any Terminating Event or Defect.

8.6. Remedies for Failed Batches and Defective Batches.

(a) If a clinical Batch of Bulk Drug Substance or Drug Product Manufactured prior to PPQ Completion is a Failed Batch or a Defective Batch, Catalent will at [**] election:

(i) Manufacture a new Batch of Bulk Drug Substance and/or Drug Product that meets the Product Requirements and procure any necessary additional Raw Materials, at [**] cost and expense, as soon as practicable (including any substitute Batches of Bulk Drug Substance necessary to Manufacture a replacement Batch of Drug Product), taking into account the availability of Raw Materials, *except* that the Manufacture of the new Batch and any necessary additional Raw Materials shall be at [**] cost and expense if [**]; or

(ii) if reasonably possible and acceptable to Sarepta for its purposes, Reprocess the Batch of Bulk Drug Substance or Drug Product and procure any necessary additional Raw Materials, at [**] cost and expense, as soon as practicable, with the goal that the Batch of Bulk Drug Substance and/or Drug Product meets the Product Requirements, *except* that the Reprocessing of the Batch and any necessary additional Raw Materials shall be at [**] cost and expense [**].

(b) Following PPQ Completion, if a Batch of Drug Product is a Failed Batch or a Defective Batch, Catalent will at [**] election:

(i) at [**] cost and expense, including the cost and expense to Manufacture substitute Bulk Drug Substance but excluding the Additional Raw Materials Cost unless otherwise provided in Section 8.6(d), Manufacture a new Batch of Drug Product that meets the Product Requirements as soon as practicable, taking into account the availability of Raw Materials; or

(ii) if reasonably possible and acceptable to Sarepta for its purposes, Reprocess the Batch of Drug Product, at [**] cost and expense, excluding the Additional Raw Materials Cost unless otherwise provided in Section 8.6(d), as soon as practicable, with the goal that the Batch of Drug Product meets the Product Requirements.

(c) Following PPQ Completion, if an [**] Batch or a Batch of Bulk Drug Substance is a Failed Batch or a Defective Batch, Catalent will at [**] election:

(iii) at [**] cost and expense, excluding the Additional Raw Materials Cost unless otherwise provided in Section 8.6(d), Manufacture a new [**] Batch or Batch of Bulk Drug Substance that meets the Product Requirements as soon as practicable, taking into account the availability of Raw Materials; or

(iv) if reasonably possible and acceptable to Sarepta for its purposes, Reprocess the Batch of Bulk Drug Substance, at [**] cost and expense, excluding the Additional Raw Materials Cost unless otherwise provided in Section 8.6(d), as soon as practicable, with the goal that the Batch of Bulk Drug Substance meets the Product Requirements.

(d) Except as provided in Section 8.6(e), the financial responsibility for the Additional Raw Materials Cost between Sarepta and Catalent for Failed or Defective Batches shall be based upon Catalent meeting a certain rate of success in the Manufacture of the Lead DMD Product. The success rate is determined on an annual basis based upon the total number of Batches for which Manufacturing is initiated by Catalent over the course of four (4) consecutive BQs (the four collective BQs each being a "**4BQ Period**"), starting with calendar year 2022. As provided for below in this Section 8.6(d), the number of Batches in each 4BQ Period for which Sarepta will pay for the Additional Raw Materials Cost will be determined in accordance with the following formula:

[**]

[**] shall pay the Additional Raw Materials Cost necessary for the supply of any substitute Bulk Drug Substance and/or Drug Product that must be replaced as required under Section 8.6(a), (b), or (c) for Failed or Defective Batches up to the number of [**].

[**]

(e) If any Sarepta Materials cause a Batch of Bulk Drug Substance or Drug Product to be a Failed Batch or Defective Batch, as determined by an investigation conducted pursuant to the Quality Agreement, such Failed Batch or Defective Batch shall not be counted as Failed or Defective Batch in the calculations provided for in Section 8.6(d) and Sarepta shall pay for the Batch as if it was Delivered (or, if terminated early, a portion of a Failed Batch based upon when it was terminated). Further Catalent will, at [**] cost and expense for any Additional Raw Materials Cost and the Sarepta Materials:

(i) Manufacture a new Batch that meets the Product Requirements as soon as practicable, taking into account the availability of Raw Materials; or

(ii) If reasonably possible and acceptable to Sarepta for its purposes, Reprocess the Batch, with the goal of the Batch meeting the Product Requirements.

Upon Delivery of the new Batch or Reprocessed Batch that meets the Product Requirements, Sarepta shall be invoiced for said Batch pursuant to the invoicing procedures set forth in Section 5.10.

(f) In the case of any Failed Batch or Defective Batch with respect to which Catalent is obligated to pay for the Sarepta Materials, Sarepta shall provide such materials to Catalent at Sarepta's actual cost to replace such materials.

(g) Within [**] following the completion of each [**] Period, Catalent shall prepare a reconciliation and provide Sarepta with a reasonably detailed report with respect to the Additional Raw Materials Costs charged to Sarepta and those that are ultimately determined to be Sarepta-Covered Additional Raw Materials Costs. In the event the amounts paid by Sarepta to Catalent for Additional Raw Materials Costs exceed the Sarepta-Covered Additional Raw Materials Costs, Catalent shall refund such excess amount to Sarepta within [**] from the date of such reconciliation or, at the election of Sarepta, promptly credit Sarepta such excess amount.

8.7. No Other Representations and Warranties. THE OBLIGATIONS OF CATALENT SET FORTH IN SECTION 8.6 ABOVE, INCLUDING TO REPLACE OR RE-PROCESS A FAILED BATCH OR A DEFECTIVE BATCH OF BULK DRUG SUBSTANCE AND/OR DRUG PRODUCT AND TO INCUR RELATED COSTS, INCLUDING CERTAIN RAW MATERIAL COSTS, SUBJECT TO THE LIMITATIONS SET FORTH IN SECTION 19.2(h) BELOW, SHALL BE, TOGETHER WITH SAREPTA'S RIGHTS UNDER SECTIONS 7.4 AND 18.6, SAREPTA'S SOLE AND EXCLUSIVE REMEDY UNDER THIS AGREEMENT FOR A FAILED BATCH OR DEFECTIVE BATCH OF BULK DRUG SUBSTANCE OR DRUG PRODUCT AND IS IN LIEU OF ANY OTHER WARRANTY, EXPRESS OR IMPLIED.

8.8. Survival. The provisions of Section 8.6 shall survive termination or expiration of this Agreement.

ARTICLE IX
DESIGNATED PERSON; BATCH RECORDS

9.1. Designated Person. Catalent shall at all times and in accordance with any applicable U.S. and/or other applicable regulations employ and designate in writing a designated person who shall have the necessary experience, qualifications, independence and authority and who will supervise the overall quality assurance of the Manufacture of Bulk Drug Substance, including those quality requirements set forth in cGMPs, this Agreement and the Quality Agreement. Such individual shall be responsible for confirming by his/her signature on the appropriate batch record that each Batch of Bulk Drug Substance and Drug Product conforms with the Product Requirements and is Manufactured in accordance with the Quality Agreement.

9.2. Certificates and Documentation. Catalent shall at all times and in accordance with relevant U.S. and/or other applicable regulations supply to Sarepta and its Affiliates (as applicable) with respect to, and in advance of, each Delivery of Bulk Drug Substance and Drug Product the relevant:

(a) copy of the Batch Record;

(b) Certificate of Compliance in accordance with the format and subject matter as set out in the Quality Agreement;

(c) Certificate of Analysis; and

(d) such quality related documentation as is required under the Quality Agreement, duly signed by the designated person, corresponding to the Batch of Bulk Drug Substance and Drug Product contained in that Delivery (the foregoing, collectively, referred to as the "**Batch Documentation**").

9.3. Release. The designated person shall be responsible for the release of each Batch of Bulk Drug Substance and Drug Product after review of the Batch Documentation (which shall be signed in accordance with the Quality Agreement) for each Batch of Bulk Drug Substance that has been produced.

ARTICLE X
CONTINUOUS IMPROVEMENT PROGRAM

10.1. Program. Catalent agrees to use its commercially reasonable efforts to identify and implement all practical areas of cost reduction and improvement relating to the performance of its obligations hereunder including the following:

(a) improvements in quality and technology and use of best practices;

(b) improvements in quality and technology and use of best practices relating to the Manufacture of Lead DMD Product and Good Manufacturing Practices in accordance with the Quality Agreement;

(c) reduction of waste associated with Manufacture of Lead DMD Product;

(d) a target [**] reduction in all costs associated with the performance of Catalent's obligations under this Agreement including all costs associated with the Manufacture and Delivery of Lead DMD Product under this Agreement;

(e) improvements in quality of service provided by Catalent to Sarepta in connection with the performance of this Agreement;

(f) processing time reduction in respect of the Manufacture of Bulk Drug Substance and Drug Product;

(g) improvements in the supply chain efficiency between Catalent and Sarepta and its Affiliates in connection with the performance of this Agreement (including order/Delivery process, Delivery procedures and transport costs where relevant as well as improving the ability to get access to formulation and filling areas close to the [**] Facility with the goal of facilitating the supply chain of the Lead DMD Product); and

(h) any other objectives agreed by the Parties from time to time (collectively, the "**Continuous Improvement Program**");

provided, that the foregoing aspects of the Continuous Improvement Program will not be implemented until Sarepta determines that no action of a Regulatory Authority is necessary prior to such implementation or, if Sarepta determines that such action is necessary, until the appropriate action(s) have been taken or the appropriate Regulatory Approval(s) have been obtained.

10.2. Meetings. Reasonably in advance of [**] under this Agreement and thereafter annually during the Term of this Agreement, the JSC shall meet to agree upon:

(a) objectives for the Continuous Improvement Program;

(b) targets for cost reduction; and

(c) the means of measuring and implementing the results of the Continuous Improvement Program.

10.3. Progress. Progress against objectives shall be measured annually in accordance with Section 10.4. Catalent will use all commercially reasonable efforts to achieve the agreed objectives and targets identified for the relevant year.

10.4. Review. The Parties shall assure that the JSC shall meet annually during the Term of this Agreement to review performance in relation to the objectives agreed pursuant to Section 10.2 and on each such occasion:

(a) Catalent shall provide Sarepta with [**]; and

(b) targets for cost reduction will be agreed between the Parties for the following [**] (which shall contain clearly defined milestones, measurement arrangements and action plans).

10.5. Implementation; Costs.

(a) Implementation of any such improvement shall be subject to mutual agreement of the Parties. Catalent shall not implement any such improvement without Sarepta's prior written consent obtained through the Specification change procedures set forth in Section 5.12, and implementation shall not occur until any necessary Regulatory Approvals have been obtained from a Regulatory Authority. [**]

(b) In the event that Sarepta brings to Catalent an improvement relating to the Manufacture of Bulk Drug Substance or Drug Product, Catalent shall negotiate in good faith with Sarepta the basis on which Catalent would implement such process improvement, including responsibility for the costs for implementing such change and any applicable changes to the Batch Price under this Agreement. In addition, in the event the Continuous Improvement Program results in a significant process or other improvement relating to the Manufacture of Bulk Drug Substance or Drug Product, whether by Catalent or through the joint effort of the Parties, any actual cost savings [**] (the "**CIP Savings**"). [**]

ARTICLE XI
REGULATORY MATTERS

11.1. Ownership of Regulatory Materials. Any and all Regulatory Materials, including Regulatory Approvals, arising under this Agreement in respect of the Lead DMD Product and the Manufacture thereof, including labeling and packaging and any Drug Master Files and Chemistry, Manufacturing and Control ("**Chemistry Manufacturing and Control**" or "**CMC**") (or equivalent) sections of any such Regulatory Materials shall be in the name of Sarepta or its Affiliate(s), and Sarepta or its Affiliate(s) shall own all right, title and interest in and to all such Regulatory Materials; provided, however, that Regulatory Materials, including Regulatory Approvals, solely relating to establishment license approvals for the Manufacture of Bulk Drug Substance and/or Drug Product shall be in the name of Catalent, and Catalent shall own all right, title and interest in and to only such Regulatory Materials, subject to and after giving effect to Sarepta's right to use such establishment license approvals in connection with its Development, Manufacturing and Commercialization activities for the Manufacture of Bulk Drug Substance and/or Drug Product.

11.2. Drug Product Regulatory Filings and Regulatory Approvals.

(a) **Sarepta General Responsibilities**. Sarepta and/or its Affiliates shall be solely responsible for the preparation of all Regulatory Materials owned by Sarepta and/or its Affiliates, and all Sarepta costs related thereto, including as may be necessary or desirable for obtaining and maintaining Regulatory Approvals owned by Sarepta and/or its Affiliates. [**] in the event of any gap between such requirements and the existing Specifications and/or Manufacturing activities, Sarepta and/or its Affiliates shall coordinate with Catalent as to any necessary Specification or other changes, the cost of which shall be borne solely by Sarepta.

Catalent shall cooperate with Sarepta and/or its Affiliates by providing information and consultation reasonably necessary for Sarepta and/or its Affiliates to determine whether any such gap exists and to identify efficient approaches to resolving any such gaps. With respect to the Lead DMD Product, prior to submitting to a Regulatory Authority for the first time Regulatory Materials that identify Catalent as the site of manufacture of the Lead DMD Product, Sarepta will obtain the prior written consent of Catalent to such identification. In addition, prior to submitting to a Regulatory Authority any changes to the manufacturing portion of any Regulatory Materials that relate to Catalent, Sarepta will obtain the prior written consent of Catalent to such change. In each case, such consent shall not be unreasonably withheld and shall be memorialized in a writing signed by authorized representatives of both Parties or in the minutes of a meeting of the JSC.

(b) **Manufacturing Approvals and Catalent Facility Related Sections**. Catalent shall be responsible for the preparation of all Regulatory Materials solely relating to establishment license approvals for the Manufacture of Bulk Drug Substance and Drug Product of Sarepta Drug Products under the Dedicated Clean Room Collaboration and this Agreement, including as may be necessary or desirable for obtaining and maintaining such establishment license approvals with respect to the Catalent Facility. [**] Subject to its rights set forth in Section 2.6 above, Catalent shall provide such Regulatory Materials with the content and in the format required by Regulatory Authorities and the applicable Quality Agreement, as well as such other content and format reasonably requested by Sarepta. To the extent that a Regulatory Approval of the Lead DMD Product in jurisdictions other than the U.S. or EU impose additional requirements on Catalent, Catalent agrees to comply with such requirements, at [**] cost and expense, provided that compliance with the additional requirements will not unreasonably disrupt Catalent's operation of the [**] Facility as a multi-customer, multi-product facility.

11.3. Regulatory Authority Communications. Sarepta shall be primarily responsible for communicating with any Regulatory Authority having jurisdiction anywhere in the world regarding the Lead DMD Product; provided, that Sarepta shall keep the JSC reasonably and timely informed of all such relevant communications regarding the Lead DMD Product or its components; and Catalent shall, where requested by Sarepta to do so, assist Sarepta in communications as they pertain to the Manufacture of Bulk Drug Substance and/or Drug Product, including but not limited to provision of documentation and other evidence, preparation for and participation in any inspection and conduct of any other activities necessary to facilitate the communications between Sarepta and the Regulatory Authority. Catalent shall be responsible for communicating with any Regulatory Authority having jurisdiction over the Catalent Facility regarding matters specifically related to the Catalent Facility; provided, that Catalent shall, as promptly as practicable but in no event later than the time frames agreed in the Quality Agreement, notify Sarepta in the event that Catalent communicates, or intends to communicate, either on its own initiative in accordance with this Agreement or as a result of such a Regulatory Authority initiating contact with Catalent that may affect or involve operations or other activities associated therewith.

11.4. Regulatory Compliance.

(a) Catalent shall promptly provide to Sarepta, [**] for its provision to the relevant Regulatory Authorities, all such documents and information as may be required from time to time by such Regulatory Authorities with respect to the Manufacture of Bulk Drug Substance

or Drug Product. In addition, Catalent shall promptly, at Sarepta's or the Regulatory Authority's request, provide such documents and information directly to the Regulatory Authorities. In the case Catalent receives a direct communication or request for information from a Regulatory Authority with respect to the Manufacture of Bulk Drug Substance, Drug Product and/or the Catalent Facility, Catalent shall give prompt written notice thereof to Sarepta and consult with Sarepta, and Sarepta shall be entitled to [**]. For the avoidance of doubt, the foregoing provisions of this Section 11.4(a) apply to any response to the findings of an inspection carried out according to Sections 11.4(b) or 11.8, to the extent such response is related to Manufacture of Bulk Drug Substance or Drug Product under this Agreement or Sarepta's pursuit of Regulatory Approval for the Lead DMD Product.

(b) Catalent shall allow and shall be responsible for handling inspections of the Catalent Facility as requested by any Regulatory Authority, the findings of which inspections to the extent they affect the Manufacture of Bulk Drug Substance or Drug Product shall promptly be made known in writing to Sarepta. Catalent shall, to the extent practicable, notify Sarepta in advance of any such inspection relating to the Manufacture of Bulk Drug Substance or Drug Product and provide Sarepta with the opportunity to attend and participate.

(c) If any Regulatory Authority requires any changes to be made with respect to the Manufacture of Bulk Drug Substance or Drug Product, Catalent shall (i) immediately notify Sarepta in accordance with the Quality Agreement; (ii) send Sarepta copies of any relevant documents delivered to it by said Regulatory Authority in accordance with the Quality Agreement; (iii) formulate an action plan with Sarepta in accordance with the Quality Agreement; and (iv) otherwise undertake such matters pursuant to Section 5.11.

(d) Catalent shall respond in accordance with the Quality Agreement to any questions of a regulatory nature relating to Bulk Drug Substance, Drug Product or their Manufacture raised either by Sarepta, its Affiliates, sub-licensees or distributors, or by a Regulatory Authority.

11.5. Adverse Event Reporting; Safety Data Exchange and Medical Inquiries.

(a) **Pharmacovigilance**. Sarepta shall be responsible for the collection, processing and submission of information related to adverse events associated with the Lead DMD Product in accordance with applicable Laws and Regulatory Acts and this Agreement. To the extent Catalent becomes aware of information related to an adverse event, it shall promptly provide such information to Sarepta and shall otherwise provide all reasonable assistance Sarepta may request in the handling of adverse event reports.

(b) **Medical Inquiries**. Sarepta shall be responsible for handling all medical questions or inquiries about the Lead DMD Product, in each case in accordance with applicable Laws, Regulatory Acts and this Agreement.

11.6. Regulatory Authority Communications Received.

(a) **General**. Catalent shall inform Sarepta as promptly as practicable but in no event later than within the time frames agreed in the Quality Agreement of notification of any action by, or notification or other information which it receives (directly or indirectly) from, any Regulatory Authority with respect to the Bulk Drug Substance, the Drug Product or the Catalent Facility which: (i) raises any material concerns regarding the safety or efficacy of the Bulk Drug Substance or Drug Product; (ii) relates to expedited and periodic reports of adverse events with respect to the Lead DMD Product; (iii) are Regulatory Warning Notices; and/or (iv) which may have an adverse impact on Regulatory Approval, Development, Manufacturing or Commercialization of the Lead DMD Product.

(b) **Cooperation**. The Parties shall reasonably cooperate with and assist each other in complying with regulatory obligations, including by each Party providing to the Parties such information and documentation which is in such Party's possession as may be reasonably necessary for a Party to prepare a response to an inquiry from a Regulatory Authority with respect to the Bulk Drug Substance, the Drug Product or the Catalent Facility.

(c) **Disclosures**. In addition to its obligations under this Agreement, Catalent shall promptly disclose to Sarepta the following regulatory information: all material notices or demands received from Regulatory Authorities in connection with the Bulk Drug Substance, the Drug Product or the Catalent Facility, including any notice, audit notice, notice of initiation by Regulatory Authorities of investigations, inspections, detentions, seizures or injunctions, a notice of violation letter (i.e., an untitled letter), warning letter, service of process or other inquiry, including that which may affect the overall compliance status of any party participating in the Manufacturing of the Lead DMD Product.

11.7. Recall, Withdrawal, or Market Notification of Product.

(a) **Notification and Determination**. If a Party receives any notice from a Governmental Authority threatening or initiating any action to remove the Lead DMD Product from the market, the Party receiving notice thereof shall notify the other Party of such communication as promptly as practicable but in no event later than within the time frames agreed in the applicable Quality Agreement.

(b) **Sarepta Responsibility for Recall of Drug Products**. Sarepta shall be responsible and shall determine whether to initiate any recall, withdrawal or market notification of Lead DMD Product, including the scope of such recall or withdrawal (e.g., a full or partial recall, or a temporary or permanent recall) or market notification; provided, however, that before Sarepta initiates a recall, withdrawal or market notification, it will inform Catalent of such action as far in advance as reasonably practicable.

(c) **General**. In the event of any such recall, withdrawal or market notification, [**] shall determine the necessary actions to be taken, and shall implement such action, and [**] shall provide reasonable assistance to [**] if required.

(d) **Cost Allocation**. All direct costs and expenses associated with implementing a recall, withdrawal or market notification with respect to Lead DMD Product shall be allocated between the Parties as follows: [**].

11.8. Regulatory Inspections & Audits.

(a) Sarepta shall have the right, [**] from time to time during the Term of this Agreement but not more than [**] (unless otherwise agreed between the Parties and subject to and after giving effect to the provisions of Section 11.8(b)), and pursuant to the procedures set forth in the Quality Agreement (e.g., advance notice and duration of inspection), to enter and inspect the Catalent Facility and any related utilities and/or services used in the Manufacture of Bulk Drug Substance and/or Drug Product in order to:

(i) carry out a cGMPs, quality and compliance audit of those parts of the Catalent Facility involved in or which could have any impact on the Manufacture of Bulk Drug Substance and/or Drug Product (including those used for storing, warehousing and/or testing and utilities); and

(ii) examine any Bulk Drug Substance and any Drug Product in inventory or otherwise stored at the Catalent Facility, to ensure compliance with the terms of this Agreement.

(b) Notwithstanding Section 11.8(a), Sarepta shall have the right to enter the Catalent Facility, subject to its compliance with the Catalent Facility's applicable health and safety requirements, at any time during normal business hours, in accordance with its rights set forth in the Quality Agreement or as otherwise necessary in Sarepta's discretion to comply with an obligation to a Regulatory Authority. In addition to the rights set out in Section 11.8(a), where any audit carried out in accordance with this Section 11.8(b) has identified any observations or negative findings then Sarepta shall have the right to carry out, upon reasonable prior notice and during normal business hours, follow up compliance audit(s).

(c) Catalent shall be solely responsible for ensuring the compliance status of any approved subcontractors used in relation to the performance of its obligations pursuant to this Agreement and the Quality Agreement. Catalent shall use commercially reasonable efforts to procure the right for Sarepta to have the same inspection rights described in this Section 11.8 at the premises of any such subcontractor. If Catalent is unable to procure such rights, it shall carry out such inspections itself on Sarepta's behalf and shall report the findings within [**] of completing the same.

(d) For the avoidance of doubt, any audits or inspections, checking or tests conducted by Sarepta or any of its servants or agents in relation to Bulk Drug Substance, Drug Product or the matters covered under this Agreement shall in no way diminish or relieve Catalent of any of its obligations hereunder.

11.9. Environmental Audit of Catalent. Catalent agrees that Sarepta (the "**Auditing Party**") shall have the right upon reasonable notice and during normal business hours, at the

Auditing Party's expense, once every year during the Term of this Agreement to conduct, or to nominate a Third Party (subject to execution of confidentiality and indemnity agreements reasonably acceptable to Catalent and the Auditing Party) to conduct, jointly or on the Auditing Party's behalf, an environmental audit of Catalent's operations at the Catalent Facility under this Agreement to monitor Catalent's compliance with applicable environmental Laws and Regulatory Acts, and with applicable environmental, health or safety guidelines; provided, however, [**]

ARTICLE XII
LIMITATIONS AND EXCLUSIVITY

12.1. Limitations.

(a) **No Unauthorized Use**. Each Party covenants that it will not use or practice any Sarepta Intellectual Property, Catalent Intellectual Property, Sarepta Arising IP, Catalent Arising IP or Confidential Information licensed, sublicensed, disclosed or otherwise made available to it by the other Party under this Agreement or the Collaboration Agreement except for the purposes expressly permitted herein. Except as explicitly set forth herein, no Party grants any license, express or implied, under any Patents, Regulatory Materials, Confidential Information or any other Intellectual Property rights, whether by implication, estoppel or otherwise.

(b) **Limited Access**. Catalent hereby covenants to and agrees with Sarepta that it and its Affiliates shall limit access to the Sarepta Materials, Sarepta Technology and Confidential Information to such employees of Catalent or its Affiliates who have a need to know or access the same.

12.2. Exclusivity.

(a) During the Term, [**].

(b) [**]

(c) [**] shall cause all its Affiliates to abide by this Section 12.2.

ARTICLE XIII
FORCE MAJEURE

13.1. Force Majeure Events. Subject to and except for Sarepta's rights of termination set forth in Section 7.4(b), no Party shall be in default under this Agreement because of any failure to perform if the failure arises from causes beyond the control and without the fault or negligence of such Party ("**Force Majeure Event**"), unless:

(a) The supplies, services or other subject matter impacted by the Force Majeure Event were obtainable from other sources through the use of commercially reasonable efforts; and

(b) The Party experiencing a Force Majeure Event preventing it from performing its obligations or duties under this Agreement failed to obtain such supplies, services or other subject matter therefrom.

13.2. Examples. Examples of these Force Majeure Events are: (1) acts of God or of the public enemy, (2) acts of any Governmental Authority in either its sovereign or contractual capacity, (3) fires, (4) floods, (5) epidemics, (6) quarantine restrictions, (7) strikes (exclusive of labor disputes that Catalent or its Affiliates have the discretion and authority to resolve), (8) freight embargoes, and (9) unusually severe weather. As used in this Article XIII, "**Default**" includes failure to make progress in the work so as to endanger performance of the Scope or Work or any Purchase Orders within the timeline mutually agreed upon between the Parties or set forth in the applicable Purchase Order, as applicable.

13.3. Process. The Party experiencing a Force Majeure Event preventing it from performing its obligations or duties under this Agreement shall promptly notify the other Party of the occurrence and particulars of such Force Majeure Event and shall provide the other Party, from time to time, with its best estimate of the duration of such Force Majeure Event and with notice of the resolution or cessation thereof. The Party so affected shall use commercially reasonable efforts to avoid or remove such causes of nonperformance as soon as is reasonably practicable. Upon resolution or cessation of the Force Majeure Event, the performance of any suspended obligation or duty under this Agreement shall promptly recommence. This Article XIII will not operate to excuse payment by a Party experiencing a Force Majeure Event of any amounts due to another Party under this Agreement.

ARTICLE XIV
CONFIDENTIALITY

14.1. Confidential Information. As used in this Agreement, the term "**Confidential Information**" means the following:

(a) any and all secret, confidential or proprietary information or Intellectual Property, including any data or materials, whether in written, oral, graphic, video, computer or other form, which is disclosed or made available by a Party or an Affiliate or Representative of such Party (the "**Disclosing Party**") to the other Party or an Affiliate or Representative of such other Party (the "**Receiving Party**") pursuant to this Agreement (including Confidential Information disclosed prior to the Original Effective Date hereof in connection with the Lead DMD Product, including any information disclosed by a Party pursuant to the Mutual Confidential Disclosure Agreement [**] between Sarepta and Catalent, the Interim Services Agreement, the Original Lead DMD Agreement or the Original Collaboration Agreement) or which arises as a result of this Agreement or the Original Lead DMD Agreement, and which: (i) if disclosed in written, graphic, electronic or other tangible form, is labeled as confidential or proprietary, (ii) if disclosed orally or visually, is identified as confidential or proprietary at the time of disclosure and is confirmed to be confidential or proprietary by the Disclosing Party in writing to the Receiving Party within [**] of such disclosure, (iii) by its nature, should reasonably be considered to be confidential or proprietary; or (iv) is specifically designated as Confidential Information herein; and

(b) any information concerning this Agreement; and

(c) includes but is not limited to that which relates to business plans, strategic plans or business methods that derive economic value from not being generally known to other persons or easily ascertainable by other persons, business policies, research, product plans, Drug Products, product pricing or product strategy, services, service pricing or service strategy, manufacturing information, actual or proposed alliance partners, actual or proposed vendors, vendor offerings and pricing, actual or proposed customers, customer usage and customer purchasing potential, employee and consulting relationship information, actual or proposed markets, sales and marketing materials, plans and methods, specifications, shop-practices, software, developments, inventions (whether or not patented), product names or marks, trade secrets, technologies, discoveries, and any other intellectual property (whether or not registered), processes, designs, drawings, engineering, hardware configuration information or finance, accounting or financial plans and forecasts, compilations, formulas, devices, methods, prototypes, techniques, procedures, protocols, programs, records, and databases.

14.2. Exceptions to Confidential Information. Confidential Information shall not include any information or materials to the extent the Receiving Party can reasonably demonstrate through its contemporaneous written records that such information or materials are or have been:

(a) part of public domain at the time of (i) its creation under this Agreement, or (ii) its receipt by the Receiving Party, or which thereafter becomes part of the public domain other than as a result of a breach of this Agreement or the obligations of confidentiality under this Agreement; or

(b) is approved in writing by the Disclosing Party for release; or

(c) independently developed by the Receiving Party or its Affiliates or Representatives without use of or reference to the Confidential Information of the Disclosing Party; or

(d) received from a Third Party who, to the knowledge of the Receiving Party, is not under any obligation of confidentiality towards the Disclosing Party with respect to such information.

The Receiving Party has the burden of proving any of the above exceptions. The Disclosing Party has the right to inspect the Receiving Party's documentary evidence upon which the Receiving Party bases its claim that Confidential Information is within any of the above exceptions.

14.3. Confidentiality Obligations. Each Party shall keep all Confidential Information received from or on behalf of another Party with the same degree of care with which it maintains the confidentiality of its own Confidential Information, but in all cases no less than a reasonable degree of care. Each Party, in their position as a Receiving Party hereunder, shall, during the Term and for [**] thereafter:

(a) not use the Disclosing Party's Confidential Information other than as strictly necessary to exercise its rights and perform its obligations under this Agreement; and

(b) maintain the Disclosing Party's Confidential Information in strict confidence and, subject to Section 14.4, not disclose the Disclosing Party's Confidential Information to any Person without the Disclosing Party's prior written consent, provided, however, the Receiving Party may disclose the Confidential Information to its Representatives who:

(i) have a need to know the Confidential Information for purposes of the Receiving Party's performance, or exercise of its rights concerning the Confidential Information, under this Agreement;

(ii) have been apprised of the obligations of confidentiality, non-disclosure, and non-use under this Agreement; and

(iii) are themselves bound by written nondisclosure agreements or ethical obligations of confidentiality at least as restrictive as those set forth in this Section 14.3, provided further that the Receiving Party shall be responsible for ensuring its Representatives' compliance with, and shall be liable for any breach by its Representatives of the confidentiality, non-disclosure and non-use obligations set forth herein.

14.4. Permitted Disclosure and Use. Notwithstanding Section 14.3, a Party may disclose Confidential Information belonging to another Party if and only to the extent such disclosure is reasonably necessary to:

(a) comply with applicable Laws, Regulatory Acts, rules, regulations, government requirements or court orders, provided that the Receiving Party shall promptly notify the Disclosing Party of its notice of any such requirements; provide the Disclosing Party a reasonable opportunity to seek a protective order or other appropriate remedy or waive its rights under this Article XIV; and disclose only the portion of Confidential Information that it is legally required to furnish;

(b) secure any Regulatory Approvals for the Sarepta Drug Products, provided that the Disclosing Party will take all reasonable steps to limit disclosure of the Confidential Information outside such regulatory agency and to otherwise maintain the confidentiality of the Confidential Information; or

(c) solely with respect to Confidential Information consisting of this Agreement and the financial aspects of this Agreement, for the presentation of or reporting to financial agencies or institutions, actual or potential investors or brokers, and their respective officers, directors, employees and representatives for the limited purpose of securing debt or equity financing and maintaining compliance with the definitive agreements executed in connection therewith; provided that any such disclosure is provided pursuant to a confidentiality agreement containing similar to or more restrictive than the terms in this Agreement and provided further that the Party exercising its rights under this Section 14.4(c) shall be responsible for ensuring such recipients' compliance with, and shall be liable for any violation by such recipients of, Catalent's confidentiality and disclosure obligations contained herein.

14.5. Notification. A Receiving Party shall notify a Disclosing Party promptly upon discovery of any unauthorized use or disclosure of a Disclosing Party's Confidential Information and will reasonably cooperate with a Disclosing Party to assist a Disclosing Party to regain possession of such Confidential Information and to prevent its further unauthorized use or disclosure.

14.6. Publicity. Except as otherwise provided in this Article XIV, each Party shall maintain the confidentiality of all provisions of this Agreement, and without the prior written consent of the other Party, which consent shall not be unreasonably withheld, no Party nor its respective Affiliates shall make any press release or other public announcement of the provisions of this Agreement to any Third Party, except for: (i) disclosures required by stock exchange regulation or any listing agreement with a national securities exchange, in which case a Disclosing Party shall provide the other Parties with [**] notice unless otherwise not practicable, but in any event no later than the time a disclosure required by such stock exchange regulation or listing agreement is made; and (ii) disclosures as may be required by applicable Laws and Regulatory Acts, including but not limited to those required by the Securities Exchange Commission and the FDA, in which case a Disclosing Party shall provide the other Party with prompt advance notice of such disclosure and cooperate with the other Party to seek a protective order or other appropriate remedy, including a request for confidential treatment in the case of a filing with the Securities and Exchange Commission. A Party may publicly disclose without regard to the preceding requirements of this Section 14.6 any information that was previously publicly disclosed pursuant to this Section 14.6.

14.7. Use of Names. Except as otherwise set forth in this Agreement, no Party shall use the name of another Party in any public announcement, press release or other public document without the written consent of such other Party; provided, however, that, subject to and after giving effect to the provisions of Sections 14.4 and 14.6, any Party may use the name of another Party in any document filed with any Regulatory Authority or Governmental Authority, including the FDA, EMA and the Securities and Exchange Commission.

14.8. Defend Trade Secrets Act Notice. The Receiving Party acknowledges, and shall inform its Representatives of, the following notice required by the Defend Trade Secrets Act: An individual will not be held criminally or civilly liable under any federal or state trade secret law for the disclosure of a trade secret that is made in confidence to a federal, state, or local government official or to an attorney solely for the purpose of reporting or investigating a suspected violation of law. Similarly, an individual will not be held criminally or civilly liable under any federal or state trade secret law for the disclosure of a trade secret that is made in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal. An individual who files a lawsuit for retaliation by an employer for reporting a suspected violation of law may disclose the trade secret to that individual's attorney and use the trade secret information in the court proceeding, if the individual files any document containing the trade secret under seal; and does not disclose the trade secret, except pursuant to court order.

ARTICLE XV
INTELLECTUAL PROPERTY

15.1. General. This Article XV provides the general terms regarding Intellectual Property and attendant rights of the Parties thereto for the Manufacture and supply of the Lead DMD Product. In the event of a conflict between the terms and conditions in this Agreement as to Intellectual Property associated with the Lead DMD Product and the terms and conditions in the Amended and Restated Collaboration Agreement, the conflicting terms and conditions in this Agreement shall control. In the event of a conflict between the terms and conditions in this Agreement as to Intellectual Property associated with the Sarepta Clean Room Suites and the terms and conditions in the Amended and Restated Collaboration Agreement, the conflicting terms and conditions in the [**] shall control.

15.2. Sarepta Materials. As between the Parties, Sarepta shall own all rights in and title to the Sarepta Materials, and all derivatives, modifications, combinations with other materials and improvements thereto, including methods for making and using the Sarepta Materials or such derivatives, modifications or improvements.

15.3. License to Catalent. Sarepta retains all right, title, and interest in and to any Sarepta Intellectual Property. In recognition of the additional process development and analytical testing work undertaken by Sarepta without the assistance of Catalent (including the reliance on or reference to Catalent Confidential Information) or outside of either the Scope of Work generally or the Process Development-Focused SOWs specifically, the Parties agree that such activities shall be defined as Sarepta Intellectual Property, notwithstanding anything to the contrary in this Agreement or the Collaboration Agreement. Nothing in this Agreement shall be construed to grant Catalent any right or license to any Sarepta Intellectual Property except as expressly set forth herein. During the Term, Sarepta hereby grants to Catalent a fully paid, non-exclusive license under any and all Sarepta Intellectual Property and Sarepta Arising IP that is necessary for the sole and limited purpose of Catalent's performance of its obligations under this Agreement, including, without limitation, the Manufacture of Batches of the Bulk Drug Substance for Sarepta.

15.4. License to Sarepta. Catalent retains all right, title, and interest in and to any Catalent Intellectual Property. Any Improvements, other than to Sarepta Technology, created or developed solely or jointly by Catalent in the course of performing the Scope of Work, the clinical and commercial supply Manufacturing of Bulk Drug Substance or Drug Product, and/or the Continuous Improvement Program that relates generally to the Development or Manufacture of drug substances or drug products, including any process, protocol, technology, Know-How or the like that applies generally to the conduct by Catalent of laboratory and manufacturing operations and activities, except to the extent that any of the same [**] and Catalent shall own all right, title and interest therein. Catalent shall provide written notice to Sarepta of any such Catalent Arising IP, as soon as possible but no later than [**] after conception or observation of the same by Catalent. Sarepta hereby assigns to Catalent all right, title, and interest in and to all such Catalent Arising IP, free and clear of all liens, claims, and encumbrances, and shall take any actions, including but not limited to the execution of documents, reasonably requested by Catalent and at Catalent's expense, to effect the purposes of the foregoing. Catalent hereby grants to Sarepta a perpetual, irrevocable, fully paid, non-exclusive, worldwide license, with the right to grant and

authorize sublicenses through multiple tiers (a) under the Catalent Arising IP and Catalent Intellectual Property, for the sole and limited purpose of [**] and (b) under the Catalent Arising IP and [**] in each case, (a) and (b), provided that [**].

15.5. Project Intellectual Property. All Intellectual Property created or developed by Sarepta or solely or jointly by Catalent in the course of performing the Scope of Work, the clinical and commercial supply Manufacturing of Bulk Drug Substance or Drug Product, and/or the Continuous Improvement Program, that [**] Sarepta Confidential Information or Sarepta Intellectual Property, shall be "**Sarepta Arising IP**" and the exclusive property of Sarepta. As such Sarepta Arising IP is created or developed, Catalent shall provide written notice to Sarepta of any such Sarepta Arising IP, as soon as possible but no later than [**] after conception or observation of the same by Catalent. Catalent hereby assigns to Sarepta all right, title, and interest in and to all such Sarepta Arising IP, free and clear of all liens, claims, and encumbrances, and shall take any actions, including but not limited to the execution of documents, reasonably requested by Sarepta and at Sarepta's expense, to effect the purposes of the foregoing.

15.6. Existing Intellectual Property. If Catalent desires to incorporate any Catalent Intellectual Property, or to Catalent's knowledge, any Third Party Intellectual Property that would require a license or assignment not otherwise provided for in this Agreement, into the Development or Manufacture of the Lead DMD Product, Catalent will provide prior written notice and seek the written approval of Sarepta concerning the same (such notice shall be separately set forth and identified as such, and not generally contained in this Agreement or the Scope Work). Upon receipt of such a notice, Sarepta shall provide a prompt written response to Catalent no later than [**] from receipt of such notice, indicating Sarepta's approval or objection to the same. [**] Upon any objection by Sarepta, the Parties will meet and confer to determine a mutually acceptable work-around to the Intellectual Property subject to the objection. If there is no suitable work-around and the Parties desire to secure the Intellectual Property, [**] shall secure a license to the Intellectual Property at [**] cost for the [**], such license to include rights to sublicense to [**] and through multiple tiers. Catalent shall indemnify Sarepta pursuant to Section 18.6 for any Intellectual Property of any Third Party incorporated into the Development or Manufacture of the Lead DMD Product for which a license has not been secured without having provided prior notice to, and received approval from, Sarepta under this Section. Absent knowledge of actual infringement or a reasonable likelihood of potential infringement (e.g., via third party notice), Catalent is under no general obligation to undertake any patent infringement or patent clearance studies or to obtain any patent opinions, or to seek or secure any licenses to Third Party Intellectual Property on Sarepta's behalf.

15.7. Sublicenses. Sarepta shall remain fully responsible to Catalent for the performance of its Sublicensees with respect to Sarepta's obligations under the terms of this Agreement. Any act or omission of a Sublicensee which would be a material breach of this Agreement if performed by Sarepta shall be deemed to be a breach by Sarepta of this Agreement susceptible to cure within the cure period specified in Section 19.1(c)(ii). In the event Sarepta has actual knowledge of a breach of any sublicense agreement as to the misuse, misappropriation, theft or breach of confidence of Catalent Intellectual Property and Catalent Arising IP, Sarepta shall promptly inform Catalent. Within [**] of notification, Sarepta shall institute an action or proceeding against the sublicensee or otherwise confirm with reasonable supporting information that the breach is no

longer occurring. If Sarepta fails to bring such an action or proceeding or stop the breaching sublicensee, or fails to confirm that the breach is no longer occurring, Catalent shall have the right, but not the obligation, to prosecute the same solely with respect to the breaching activities. Sarepta shall, [**] reasonably cooperate with Catalent in such action at [**] expense.

15.8. Patent Filings; Cooperation. The Parties agree to cooperate in the preparation, filing, prosecution and maintenance of all Patents disclosing or claiming the Sarepta Arising IP or the Catalent Arising IP (collectively, the "**Arising IP Patents**"), including obtaining and executing necessary powers of attorney and assignments by the named inventors, providing relevant technical reports to the filing Party concerning Inventions disclosed in such Arising IP Patents, obtaining execution of such other documents which are needed in the filing and prosecution of such Arising IP Patents. The Parties shall cooperate reasonably in the prosecution of all Arising IP Patents.

15.9. Notice and Defense of Third-Party Infringement Claims. The Parties acknowledge that successful completion of Catalent's obligations under this Agreement may require a license to Third Party Intellectual Property.

(a) If Sarepta determines that a license to Third Party Intellectual Property (including Third Party Intellectual Property of other Sarepta suppliers) is required for Catalent's use of Sarepta Intellectual Property in order to Manufacture the Lead DMD Product, [**] will be responsible for obtaining and maintaining such license, which license will extend to [**] for the Manufacturing necessary hereunder.

(b) If Catalent determines that a license to Third Party Intellectual Property (excluding Third Party Intellectual Property of other Sarepta suppliers) is required for Catalent's use of Catalent Intellectual Property in order to Manufacture the Lead DMD Product, [**] will be responsible for obtaining and maintaining such license; provided, however, [**]. In the event either Party is put on notice by a Third Party of alleged infringement of such Third Party's Intellectual Property arising solely from Catalent Intellectual Property, as used by [**] in performing the Manufacturing, such Party will promptly inform the other Party of such notification, including furnishing a copy of such notification (or those portions of the notification directly pertaining to same). Catalent and Sarepta together will promptly investigate such notice, and if deemed credible by Catalent, seek to resolve the same with such Third Party in a manner that allows for the manufacture of the Lead DMD Product and exploitation, including the commercial sale, of the same. Subject to the foregoing, [**] will assume the costs of resolution, including any license fees and costs associated with litigation, associated with claims of infringement of Third Party Intellectual Rights arising solely from [**] Intellectual Property.

15.10. Patent Term Extensions. As between the Parties, Sarepta shall have the exclusive right, but not the obligation, to seek Patent Term Extensions (including any supplemental protection certificates and the like available under applicable Laws) in relation to Regulatory Approval of a Lead DMD Product. Catalent and Sarepta shall cooperate in connection with all such activities. [**]

15.11. Product Trademarks. Sarepta shall in its sole discretion select trademarks under which it shall Commercialize the Lead DMD Product.

ARTICLE XVI
AFFILIATES AND SUBCONTRACTORS

16.1. Performance by Affiliates. The Parties recognize that each may perform some or all of its obligations under this Agreement through Affiliates; provided, however, that each Party shall remain responsible for and be a guarantor of the performance by its Affiliates and shall cause its Affiliates to comply with the provisions of this Agreement in connection with such performance, and provided further, that Catalent and its Affiliates may not perform any Development, Manufacturing or Commercialization activities with respect to any Sarepta Drug Product at a facility other than the Catalent Facilities as defined herein without Sarepta's prior written consent. Each Party hereby expressly waives any requirement that the other Party exhaust any right, power or remedy, or proceed against an Affiliate, for any obligation or performance hereunder prior to proceeding directly against such Party. Wherever in this Agreement a Party delegates responsibility to Affiliates, such Party agrees that such entities may not make decisions inconsistent with this Agreement, amend the terms of this Agreement or act contrary to its terms in any way.

16.2. Performance by Subcontractors. Catalent shall have the right to subcontract a limited portion of its rights and obligations hereunder at any given time during the Term, but only after obtaining Sarepta's prior written consent, not to be unreasonably withheld; provided, however, that with respect to each such subcontract, [**]. No sublicense rights or licenses may be extended to any such permitted subcontractors of Catalent. However, it is acknowledged and understood by the Parties that the license grants provided to Catalent under this Agreement cover the activities relating to the performance of this Agreement of permitted subcontractors of Catalent and other Persons duly engaged by Catalent as contemplated hereunder. Any references in this Agreement to a "permitted" or "approved" subcontractor of Catalent shall mean those subcontractors of Catalent who satisfy all of the requirements set forth in this Section 16.2. Catalent's subcontractors approved by Sarepta at the outset of this Agreement are listed in **Exhibit C**.

ARTICLE XVII
RECORDS AND AUDITS

17.1. Records and Information.

(a) **General**. Catalent shall maintain up to date accurate records of all material activities conducted by or on behalf of it under this Agreement as well as may be required in order to comply with cGMPs, applicable Laws and Regulatory Acts and the Quality Agreement, and including in respect of all data and other information pertaining to the Manufacture of the Bulk Drug Substance and Drug Product (which records shall include, as applicable, books, records, reports, charts, graphs, comments, computations, analyses, recordings, photographs, computer programs and documentation thereof) (hereinafter the "**Catalent Records**").

(b) **Access to Records**. Sarepta and its agents or designees shall have the right: (i) at its expense, not more than [**] per calendar year, to review Catalent Records, at reasonable times, upon written request, relating to the activities undertaken by Catalent under this Agreement; and (ii) subject to Section 11.2(a), to reference any Catalent-owned Regulatory Materials as required for any submission to a Regulatory Authority.

17.2. Financial Records; [**].

(a) **Financial Records**. Catalent and any approved subcontractors shall keep full, true and accurate records and books of account containing all particulars that may be reasonably necessary for the purpose of confirming the accuracy of, and calculating, as applicable, all payments due or sought in relation to the activities under this Agreement and any other records reasonably required to be maintained with respect to Catalent's obligations under this Agreement (such records being "**Catalent Financial Records**"), and Catalent shall maintain complete and accurate records in sufficient detail to permit Sarepta to confirm the accuracy of all costs and any other amounts payable or otherwise reimbursable under this Agreement, in each case for a minimum period of [**] or such longer period as required by applicable Laws.

(b) **[**]**

ARTICLE XVIII
REPRESENTATIONS AND WARRANTIES

18.1. Sarepta and Catalent Mutual Representations and Warranties. Sarepta and Catalent each hereby represent, warrant and covenant to one another as follows, as of the Effective Date:

(a) **Corporate/Company Existence and Power**. It is a company or corporation duly organized, validly existing, and in good standing under the Laws of the jurisdiction in which it is incorporated, and has full company or corporate power and authority and the legal right to own and operate its property and assets and to carry on its business as it is now being conducted and as contemplated in this Agreement, including the right to grant the licenses granted by it hereunder (except as provided in Section 18.1(d)).

(b) **Authority and Binding Agreement**.

(i) It has the company or corporate power and authority and the legal right to enter into this Agreement and perform its obligations hereunder,

(ii) It has taken all necessary company or corporate action on its part required to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder, and

(iii) This Agreement has been duly executed and delivered on behalf of such Party, and constitutes a legal, valid, and binding obligation of such Party that is

enforceable against it in accordance with its terms, except as enforcement may be affected by bankruptcy, insolvency or other similar Laws and by general principles of equity.

(c) **No Conflicts**. The execution, delivery and performance of this Agreement by it does not (i) conflict with any agreement, instrument or understanding, oral or written, to which it is a party and by which it may be bound or (ii) violate any Laws of any Governmental Authority having jurisdiction over it.

(d) **All Consents and Approvals Obtained**. Except with respect to Regulatory Approvals for the Development, Manufacturing or Commercialization of the Lead DMD Product that are not required at the time of execution of this Agreement or as otherwise expressly noted in this Agreement, (i) all necessary consents, approvals and authorizations of, and (ii) all notices to, and filings by such Party with, all Governmental Authorities and other Persons required to be obtained or provided by such Party as of the Effective Date in connection with the execution, delivery and performance of this Agreement have been obtained and provided.

(e) **Compliance with Law**. The Parties shall perform all of their respective obligations under this Agreement in full compliance with all applicable Laws and Regulatory Acts.

18.2. Mutual Covenants; No Debarment. No Party shall use in any capacity, in connection with its Development, Manufacture or Commercialization of a Drug Product hereunder, any Person who has been debarred pursuant to Section 306 of the FD&C Act (or similar Law outside of the U.S.), or who is the subject of a conviction described in such section, and each Party shall inform the other Party in writing immediately if it or any Person who is performing services for such Party hereunder is debarred or is the subject of a conviction described in Section 306 (or similar Law outside of the U.S.), or if any action, suit, claim, investigation or legal administrative proceeding is pending or, to such Party's knowledge, is threatened, relating to the debarment of such Party or any Person used in any capacity by such Party in connection with its Development, Manufacture or Commercialization of a Drug Product hereunder.

18.3. Additional Representations, Warranties and Covenants by Catalent. Catalent represents, warrants and covenants to each other Party as follows:

(a) As of the Effective Date, to the best of Catalent's knowledge, the use of Catalent Intellectual Property in the performance of activities contemplated under this Agreement as of the Effective Date shall not infringe any Intellectual Property of Third Parties. Catalent covenants to Sarepta that it shall promptly notify Sarepta in writing should it become aware of any claims asserting such infringement.

(b) As of the Effective Date, Catalent is a wholly-owned subsidiary of CPS.

(c) To the best of Catalent's knowledge, after reasonable inquiry and investigation, (i) there is no "material" claim, action, suit, proceeding or governmental investigation ("**Action**") of any nature pending or threatened against or by Catalent; and (ii) no

event has occurred or circumstances exist that may give rise to, or serve as a basis for, any such Action. An Action is "material" herein if it could result in a final claim, judgment or penalty [**].

(d) Catalent has complied, and is now complying, with all applicable Law and all terms of the [**] lease relative to its use and improvement of the [**] Facility. Catalent shall hold, as and when necessary during the Term of this Agreement, all licenses, permits and similar authorizations required by any Regulatory Authority for Catalent to perform its obligations under this Agreement.

(e) The Batches Delivered by Catalent to Sarepta under this Agreement shall be free of any liens, security interests or other encumbrances.

(f) The Batches Delivered by Catalent to Sarepta under this Agreement shall have been manufactured in accordance with applicable Law and in conformity with the Specifications and shall not be adulterated, misbranded, or mislabeled within the meaning of applicable Law; provided that Catalent shall not be liable for defects attributable to Sarepta Materials or materials provided by or on behalf of Sarepta, other than by Catalent or its Affiliates, with respect to Batches subsequent to delivery (including, in each case to the extent applicable, artwork, advertising, and labeling).

18.4. Additional Representations, Warranties and Covenants of Sarepta.

(a) Sarepta covenants to Catalent that Sarepta shall comply with all applicable Laws in its use of the Lead DMD Product as well as Bulk Drug Substance Delivered to it under this Agreement.

18.5. No Other Representations or Warranties. EXCEPT AS EXPRESSLY STATED IN THIS AGREEMENT, NO REPRESENTATIONS OR WARRANTIES WHATSOEVER, WHETHER EXPRESS OR IMPLIED, INCLUDING ANY IMPLIED REPRESENTATION OR WARRANTY WITH RESPECT TO [**] AND, EXCEPT AS EXPRESSLY STATED IN THIS AGREEMENT, ALL REPRESENTATIONS AND WARRANTIES, WHETHER ARISING BY OPERATION OF LAW OR OTHERWISE, ARE HEREBY EXPRESSLY EXCLUDED.

18.6. Catalent Indemnity. Catalent shall defend, indemnify and hold harmless Sarepta and its Affiliates, and their respective directors, manager, officers, employees, contractors, agents and assigns (each, a "**Sarepta Indemnitee**") from and against any and all liabilities, losses, costs and expenses (collectively, "**Loss**") suffered or incurred by them in connection with any claim brought by a Third Party that arises or is alleged to arise from or in connection with:

[**]

except to the extent in each case that the Loss in question resulted from the negligence or willful misconduct of, or breach of this Agreement by, a Sarepta Indemnitee or any of its or their Representatives.

18.7. Sarepta Indemnity. Sarepta shall defend, indemnify and hold harmless Catalent and its Affiliates, and their respective directors, manager, officers, employees, contractors, agents and assigns (each, a "**Catalent Indemnitee**") from and against any and all Loss suffered or incurred by them in connection with any claim brought by a Third Party that arises or is alleged to arise from or in connection with:

[**]

except to the extent in each case that the Loss in question (i) resulted from the negligence or willful misconduct of, or breach of this Agreement by, a Catalent Indemnitee or any of its or their Representatives, or (ii) resulted from any activities for which Catalent is obligated to indemnify a Sarepta Indemnitee pursuant to Section 18.6(b).

18.8. Indemnification Procedures. The Person or Persons claiming indemnity under this Article XVIII (the "**Indemnified Party**") shall give written notice to the Party from whom indemnity is being sought (the "**Indemnifying Party**") promptly after learning of such claim. The Indemnified Party shall provide the Indemnifying Party with reasonable assistance, at the Indemnifying Party's expense, in connection with the defense of the claim for which indemnity is being sought. The Indemnifying Party shall have the right, but not the obligation, to assume and conduct the defense of the claim with counsel of its choice; provided, the Indemnified Party may participate in and monitor such defense with counsel of its own choosing at its sole expense. The Indemnifying Party shall not settle any claim without (a) first consulting with the Indemnified Party, and (b) obtaining the prior written consent of the Indemnified Party, not to be unreasonably withheld or delayed, unless the settlement involves only the payment of money. The Indemnified Party shall not settle or compromise any such claim without (x) first consulting with the Indemnifying Party, and (y) obtaining the prior written consent of the Indemnifying Party. If the Indemnifying Party does not assume and conduct the defense of the claim as provided above, (i) the Indemnified Party may, using counsel of its choice, defend against such claim in any manner the Indemnified Party may deem reasonably appropriate, and (ii) the Indemnifying Party shall remain responsible to indemnify the Indemnified Party as provided in this Article XVIII, provided, that in such instance such indemnity shall also include the reasonable legal fees and reasonable Out-of-Pocket Costs incurred by the Indemnified Party in connection with so defending itself in the absence of a defense being provided by the Indemnifying Party.

18.9. Insurance.

(a) At all times during the Term of this Agreement (including any post-termination period in which Catalent is completing its performance under a purchase order accepted prior to termination, each Party, and, to the extent applicable, its approved subcontractors, shall obtain and maintain with insurers [**] or higher at all times as of and after the date of this Agreement, at [**] cost and expense: (i) general liability insurance with a per occurrence limit of [**] or equivalent and an annual aggregate limit of [**] or equivalent; (ii) Products and Completed Operations Liability Insurance with a per occurrence limit of not less than [**] or equivalent covering each Party's own operations arising out of or connecting with this Agreement, providing coverage for bodily injury and property damage claims; and (iii) workers' compensation as required by all applicable laws and employer's liability coverage with a limit of not less than [**].

If any such policy is replaced, each Party agrees to purchase tail coverage or ensure that the new policy has a retroactive date that is consistent with the start of any work under a scope of work and that such Party will continue to be covered on the replacement policy. Each Party may self-insure all or any portion of the required insurance as long as, together with its Affiliates, its US GAAP net worth is greater than [**] or equivalent or its annual EBITDA (earnings before interest, taxes, depreciation and amortization) is greater than [**] or equivalent. Waivers of subrogation and additional insured status obligations will operate the same whether insurance is carried through third parties or self-insured. Upon the other Party's written request from time to time, each Party shall promptly furnish to the other Party a certificate of insurance or other evidence of the required insurance. Without limiting the foregoing, Catalent shall obtain and maintain all risk property insurance coverage which includes business interruption.

ARTICLE XIX
TERMINATION AND SURVIVAL

19.1. Term and Termination.

(a) The term of this Agreement shall begin on the Effective Date and continue until the earlier of (i) December 31, 2028, or (ii) termination in accordance with this Section 19.1 (the "**Term**"). The Parties may extend the Term by mutual written agreement.

(b) Sarepta shall have the right to terminate this Agreement by giving written notice to Catalent in the event of any of the following:

(i) For failure to perform, if Catalent fails to deliver at least [**] of the Batches ordered by Sarepta [**]

(ii) The Lead DMD Product subject to this Agreement (A) undergoes a market withdrawal, or (B) otherwise is determined by Sarepta or a Regulatory Authority to have material safety risks, or (C) Sarepta or a Regulatory Authority identifies sufficient questions regarding efficacy or substantial Manufacturing concerns, in each case (A) through (C), leading to the cessation or termination of Development, Manufacture or Commercialization of, or seeking Regulatory Approval for, the Lead DMD Product; or

(iii) Catalent undertakes or experiences [**]; or

(iv) If Sarepta has, in good faith, exhausted all reasonable remedies to resolve a patent dispute and a court or other competent authority (A) issues a final decision that Catalent Intellectual Property reasonably necessary to Manufacture the Lead DMD Product, or Sarepta Technology, infringes a valid and enforceable Patent held by a Third Party, or (B) grants an injunction that renders Sarepta unable to sell the Lead DMD Product; or

(v) Pursuant to the rights granted Sarepta in Section 7.4(b).

(c) Either Party hereto shall have the right to terminate this Agreement by giving the other Party written notice in the event of any of the following:

(i) In the event that (i) such other Party files in any court or agency pursuant to any statute or regulation of any jurisdiction a petition in bankruptcy or insolvency or for reorganization or similar arrangement for the benefit of creditors or for the appointment of a receiver or trustee of such other Party or substantially all of its assets, (ii) such other Party is served with an involuntary petition against it in any insolvency proceeding and such involuntary petition has not been stayed or dismissed within [**] of its filing, or (iii) such other Party makes an assignment of substantially all of its assets for the benefit of creditors; or

(ii) If the other Party is in material breach of this Agreement, provided that if the breach is capable of cure (1) the non-breaching Party shall first provide [**] prior written notice and an opportunity to cure to the breaching Party and (2) in the event the breach is not cured within such [**] period, the breaching Party has not diligently pursued an acceptable cure and provided a reasonable plan of proposed actions and schedule for completing such cure outside the [**] period that the non-breaching Party agrees, in its sole discretion, is reasonably likely to allow for cure in a sufficient and timely enough manner; or

(iii) The other Party is suspended or debarred by FDA or the United States government.

19.2. Effect of Termination.

(a) The Party making notification of termination shall specify in the termination notice the effective date of the termination (the "**Termination Date**"), which, in the event of termination pursuant to Section 19.1(c)(ii), shall be no sooner than the date that is [**] after notification of the breach if such breach is capable of cure.

(b) If this Agreement is terminated by Catalent pursuant to Section 19.1(c)(ii), the economic consequences will be solely as follows (except, in the case of termination for breach, to the extent additional remedies are available under applicable Law): [**].

(c) If this Agreement is terminated by Sarepta pursuant to Sections 19.1(b)(ii), (iii) or (iv), the economic consequences will be solely as follows: [**].

(d) Under any termination of this Agreement, except for any termination by Sarepta pursuant to Section 19.1(c) or, in the event that Catalent Intellectual Property reasonably necessary to Manufacture the Lead DMD Product infringes a valid and enforceable Patent held by a Third Party, under Section 19.1(b)(iv), and in addition to amounts paid pursuant to Sections 19.2(b) and (c), Catalent shall [**]. Additionally, under any termination of this Agreement, the JSC shall review and approve the disposition of any Raw Materials procured and stored for the Manufacture of Bulk Drug Substance or Drug Product under this Agreement, [**].

(e) If this Agreement is terminated by Sarepta pursuant to Section 19.1(c), Catalent's obligation under Section 12.2 shall continue for a period no longer than the earlier of ([**].

(f) Except as otherwise provided in Sections 19.2(b) and 19.2(c), payments owed to Catalent in connection with a termination of this Agreement shall be made within [**] after the later of the Termination Date or the receipt of an invoice for the same. Payments under Section 19.2(d) shall be made to Catalent within [**] following the submission to Sarepta of an accounting of all non-cancellable fees and expenses incurred by Catalent and a corresponding invoice.

(g) If this Agreement is terminated by Sarepta pursuant to Sections 19.1(c), Section 19.1(b)(i) or 19.1(b)(iv), Catalent shall, within [**] of the Termination Date, refund any deposits for those portions of any outstanding Scope of Work that have not been completed, return any amounts advanced for cancellable costs, and deliver to Sarepta all inventory of Sarepta Materials and any other Raw Materials for which Sarepta has paid; and Sarepta shall be relieved of all further purchase and payment obligations under this Agreement.

(h) EXCEPT AS EXPRESSLY PROVIDED IN [**], CATALENT SHALL HAVE NO LIABILITY UNDER THIS AGREEMENT FOR ANY AND ALL CLAIMS FOR LOST, DAMAGED OR DESTROYED SAREPTA MATERIALS, WHETHER OR NOT SUCH SAREPTA MATERIALS ARE USED IN THE SERVICES OR INCORPORATED INTO A PRODUCT. [**], CATALENT'S TOTAL LIABILITY IN ANY [**] PERIOD FOR LOST OR DAMAGED SAREPTA MATERIALS, WHETHER OR NOT SUCH SAREPTA MATERIALS ARE USED IN THE SERVICES OR INCORPORATED INTO A PRODUCT, IS LIMITED TO THE LESSER OF [**]. [**] CATALENT SHALL HAVE NO LIABILITY UNDER THIS AGREEMENT FOR ANY AND ALL CLAIMS FOR LOST, DAMAGED OR DESTROYED RAW MATERIALS, WHETHER OR NOT SUCH RAW MATERIALS ARE USED IN THE SERVICES OR INCORPORATED INTO A PRODUCT. SAREPTA AGREES THAT TO THE FULLEST EXTENT PERMITTED BY LAW, CATALENT'S LIABILITY TO SAREPTA FOR ANY AND ALL INJURIES, CLAIMS, LOSSES, EXPENSES, OR DAMAGES, WHATSOEVER, ARISING OUT OF OR IN ANY WAY RELATED TO THE ACTIVITIES OF THIS AGREEMENT, FROM ANY CAUSE OR CAUSES INCLUDING, BUT NOT LIMITED TO, BREACH OF CONTRACT, NEGLIGENCE, ERRORS, OMISSIONS OR STRICT LIABILITY, SHALL NOT EXCEED [**], EXCEPT THAT THE FOREGOING LIMITATION OF LIABILITY SHALL NOT APPLY TO LIABILITY THAT IS SUBJECT [**]. IN NO EVENT WILL EITHER PARTY BE LIABLE TO THE OTHER PARTY FOR ANY INDIRECT, SPECIAL, CONSEQUENTIAL, PUNITIVE OR EXEMPLARY DAMAGES (INCLUDING, BUT NOT LIMITED TO, DAMAGES BASED UPON LOST PROFITS, RELIANCE OR EXPECTATION, BUSINESS INTERRUPTION, LOST BUSINESS, OR LOST SAVINGS) FOR ANY ACTS OR FAILURE TO ACT UNDER THIS AGREEMENT, INCLUDING ANY TERMINATION OF THIS AGREEMENT IN ACCORDANCE WITH ITS TERMS, EVEN IF SUCH PARTY HAS BEEN ADVISED OF THE POSSIBLE EXISTENCE OF SUCH DAMAGES. The limitations of liability reflect the allocation of risk between the Parties. The limitations specified in this Section 19.2(h) will survive and apply even if any limited remedy specified in this Agreement is found to have failed of its essential purpose.

(i) In the event of termination or expiration of this Agreement, in addition to any rights or obligations that by their terms are intended to survive the Term of this Agreement, the following provisions shall survive such termination: Section 4.7 (solely with regard to payment obligations arising prior to and unpaid as of the date of expiration or termination, including but not limited to any obligation to purchase that Catalent cannot cancel), Section 5.10 (solely with regard to payment obligations arising prior to and unpaid as of the date of expiration or termination, including but not limited to any obligation to purchase that Catalent cannot cancel), Sections 11.5- 11.7, Section 12.1, Section 14.1 through Section 14.4 (in each case, for five years following termination or expiration), Section 14.6, Section 15.1, Section 15.2, Section 15.4, Section 15.5, Section 15.6, Section 15.7, Section 15.8, Section 15.10, Section 15.11, Sections 18.3 through 18.9, Section 19.2, Article XX, and Article XXI (except for Section 21.8). Termination or expiration of this Agreement for any reason will not relieve the Parties of any liability accruing prior thereto and will be without prejudice to the rights and remedies of any Party with respect to any antecedent breach of the provisions of this Agreement.

(j) Each Party acknowledges that, in the event of termination and unless otherwise agreed to by Sarepta, Catalent shall promptly return or destroy, as directed by Sarepta, any Sarepta Materials as well as Sarepta Technology, and each Party shall promptly return to the other Party or destroy (as such other Party may direct) all data and documents in any form comprising or containing any Confidential Information of the other Party, except that each Party may retain: (a) one copy of the other Party's Confidential Information in secure legal archives for evidentiary purposes only and (b) a copy of computer records or files containing such Confidential Information that have been created pursuant to automatic archiving or back-up procedures that cannot reasonably be deleted (collectively, "**Retained Copies**"), provided, however, that any such Retained Copies will be kept confidential by the Receiving Party in accordance with the terms and provisions of this Agreement for as long as the Receiving Party is in possession of the Retained Copies, and provided further, that in the event of a termination of this Agreement by Sarepta in connection with a Change in Control, all Retained Copies of Sarepta Confidential Information must be returned or destroyed by Catalent, at Sarepta's election, within [**]. In addition, Catalent shall provide a written certification to Sarepta that (i) Catalent and its subcontractors have satisfied their confidentiality and recording obligations in all respects and (ii) all Sarepta Technology and copies thereof on any media in possession of Catalent, any of its employees or contractors have been destroyed or returned to Sarepta, such certification to be signed by a duly authorized officer of Catalent.

ARTICLE XX
DISPUTE RESOLUTION

20.1. Disputes. The Parties recognize that, from time to time, disputes may arise as to certain matters which relate to a Party's rights and/or obligations in connection with this Agreement. It is the objective of the Parties to establish procedures to facilitate the resolution of disputes arising under this Agreement in an expedient manner by mutual cooperation and without resort to litigation. To accomplish this objective, the Parties agree to follow the procedures set forth in this Article XX to resolve any controversy or claim arising out of, relating to or in connection with this Agreement prior to the pursuit of litigation.

20.2. Dispute Resolution.

(a) In the event of a dispute under this Agreement, the Parties will refer the dispute to their respective designated executive officers for discussion and resolution, who shall attempt in good faith to resolve such dispute. If such executive officers are unable to resolve such a dispute within [**] of the dispute being referred to them, either Party shall be free to initiate the arbitration proceedings outlined in Section 20.2(b).

(b) Any dispute arising out of or relating to this contract, including the breach, termination or validity thereof, which is not resolved in accordance with Section 20.2(a) or subject to Section 20.3 shall be finally resolved by arbitration in accordance with the [**] (the "**Administered Rules**" or "**Rules**"). The arbitration shall be governed by the Federal Arbitration Act, 9 U.S.C. §§ 1 et seq., and judgment upon the award rendered by the arbitrator(s) may be entered by any court having jurisdiction thereof. The place of the arbitration shall be [**]. [**] attorney's fees, costs, and disbursements arising out of the arbitration, and shall pay [**] share of the fees and costs of the arbitrator.

20.3. Patent and Trademark Dispute Resolution. Any dispute, controversy or claim relating to the scope, validity, enforceability or infringement of any Patent rights covering the Manufacture, use or sale of any product or technology or of any trademark rights relating to any product or technology shall be submitted to a court of competent jurisdiction or patent office in which such Patent or trademark rights were granted or arose.

20.4. Injunctive Relief. Nothing herein may prevent a Party from seeking a preliminary injunction or temporary restraining order, in any court of competent jurisdiction, so as to prevent any confidential information from being disclosed in violation of an applicable confidentiality agreement entered into by the Parties or to prevent the threat of imminent harm.

20.5. Continued Performance. Unless otherwise agreed in writing, the Parties will continue to provide service and honor all other commitments under this Agreement during the course of dispute resolution pursuant to the provisions of Article XX, except as provided for in Section 2.6 or to the extent such commitments are the subject of such dispute, controversy or claim.

ARTICLE XXI
MISCELLANEOUS

21.1. Assignment; Binding Effect. This Agreement may not be assigned or otherwise transferred by either Party without the prior written consent of the other Party; provided, however, Sarepta may, without such consent, assign this Agreement (i) to any Affiliate; (ii) in connection with the transfer or sale of all or substantially all of the assets to which this Agreement relates; or (ii) in the event of the merger, reorganization or consolidation of Sarepta, provided that Sarepta will provide Catalent with prompt written notice of such assignment.

21.2. Expenses. Except as expressly specified herein, [**] expenses with respect to this Agreement.

21.3. Notices. All notices, requests, claims, demands and other communications hereunder shall be in writing and shall be deemed to have been duly given (a) when received if delivered personally, (b) when transmitted by e-mail (with confirmation of successful transmission and with a duplicate copy directed pursuant to the methods set forth in (c) or (d) below), (c) upon receipt, if sent by registered or certified mail (postage prepaid, return receipt requested) and (d) the day after it is sent, if sent for next-day delivery to a domestic address by overnight mail or courier, to the Parties at the following addresses:

If to Sarepta Sarepta Therapeutics Three, LLC

[**]

with a copy to (which shall not constitute notice to Sarepta):

Wilson Sonsini Goodrich & Rosati PC

[**]

If to Catalent Catalent Maryland, Inc.

[**]

with a copy to:

Catalent Pharma Solutions, LLC
[**]

provided, however, that if any Party shall have designated a different address by notice to the others, then to the last address so designated.

21.4. Severability. If any term, provision, covenant or restriction of this Agreement is held by a court of competent jurisdiction or other authority to be invalid, void, unenforceable or against its regulatory policy such determination shall not affect the enforceability of any others or of the remainder of this Agreement; and in connection with such term, provision, covenant or restriction of this Agreement which is held invalid, void, unenforceable or against regulatory policy, the Parties shall negotiate in good faith with a view to the substitution therefor of a suitable and equitable solution in order to carry out, so far as may be valid and enforceable, the intent and purpose of such invalid term, provision, covenant or restriction and, absent any agreement by the

Parties, such court of competent jurisdiction or other authority shall substitute therefore such term, provision, covenant or restriction as is legal, valid and enforceable but otherwise similar to the invalid term, provision, covenant or restriction.

21.5. Entire Agreement. This Agreement may not be amended, supplemented or otherwise modified except by an instrument in writing signed by the Parties hereto. This Agreement, inclusive of the Quality Agreement and Amended and Restated Collaboration Agreement, all which are incorporated by reference, contain the entire agreement of the Parties hereto with respect to the Manufacture of the Lead DMD Product, superseding all negotiations, prior discussions and agreements made prior to the date hereof.

21.6. Waiver. The failure of any Party to enforce any condition or part of this Agreement at any time shall not be construed as a waiver of that condition or part, nor shall it forfeit any rights to future enforcement thereof. No waiver of any provision of this Agreement will be valid unless made in writing and signed by the Party to which such performance is due.

21.7. Governing Law. This Agreement (including any claim or controversy arising out of or relating to this Agreement) shall be governed and construed by the Laws of the State of New York without regard to conflict of law principles that would result in the application of any Law other than the Laws of the State of New York. The United Nations Convention on Contracts for the International Sale of Goods shall not apply to this Agreement.

21.8. Further Assurances. In order to carry out the provisions of this Agreement, give effect to the transactions contemplated hereby, and to defend claims or enforce rights with respect to Third Parties, the Parties shall from time to time at the reasonable request and expense of the other Party, furnish the other Party such further information, assurances or access, execute and deliver such additional documents, instruments and conveyances, and take such other actions and do such other things, as may be reasonably necessary or appropriate.

21.9. Headings. The headings of the Articles, Sections, subsections, Schedules and Exhibits of this Agreement are inserted for convenience only and shall not be deemed to constitute a part hereof.

21.10. Counterparts. This Agreement may be signed in any number of counterparts, each and every one of which shall be considered one and the same agreement and shall become effective when a counterpart hereof shall have been signed by each of the Parties and delivered to each of the other Parties, notwithstanding variations in format or file designation which may result from the electronic transmission, storage and printing of copies of this Agreement from separate computers or printers. Facsimile signatures and signatures transmitted via PDF shall be treated as original signatures.

21.11. Construction. The language in all parts of this Agreement shall be construed, in all cases, according to its fair meaning. The Parties acknowledge that each Party and its counsel have reviewed and revised this Agreement and that any rule of construction to the effect that any ambiguities are to be resolved against a drafting Party shall not be employed in the interpretation of this Agreement.

21.12. Interpretation.

(a) When a reference is made in this Agreement to an Article, Section, Exhibit, Schedule, Recital or Preamble, such reference is to an Article, Section, Exhibit, Schedule, Recital or Preamble of or to this Agreement unless otherwise indicated.

(b) The words “hereof,” “herein,” “hereto” and “hereunder” and words of similar import, when used in this Agreement, shall refer to this Agreement as a whole, including the Exhibits and Schedules, and not to any particular provision of this Agreement.

(c) The terms defined in the singular have a comparable meaning when used in the plural, and vice versa.

(d) Words of one gender include the other gender.

(e) References to a Person are also to its successors and permitted assigns.

(f) The term “Dollars” and “$” means United States Dollars.

(g) The word “including” means “including without limitation” and the words “include” and “includes” have corresponding meanings.

(h) The word “or” shall not be exclusive but rather shall have the inclusive meaning commonly associated with “and/or.”

(i) References herein to an agreement, law or regulation include such agreement, law or regulation as amended, restated, supplemented, or otherwise modified from time to time unless otherwise specified.

21.13. Relationship of the Parties. This Agreement and the Amended and Restated Collaboration Agreement are not intended by the Parties to constitute or create a joint venture, pooling arrangement, partnership, or formal business organization of any kind, and the rights and obligations of the Parties shall be only those expressly set forth herein and therein. No Party will have any right, power or authority, nor will they represent themselves as having any authority to assume, create or incur any expense, liability or obligation, express or implied, on behalf of another Party, or otherwise act as an agent for another Party for any purpose.

* * * * * * * * * * *

IN WITNESS WHEREOF, the Parties hereto have caused this Amended & Restated Manufacturing and Supply Agreement to be executed by their respective duly authorized officers as of the date first above written.

SAREPTA THERAPEUTICS THREE, LLC.

By: /s/ Adam Hopkin

Name: Adam Hopkin

Title: Manager

CATALENT MARYLAND, INC.

By: /a/ Manja Boerman

Name: Manja Boerman

Title: President – BioModalities

[Signature page to Amended and Restated Manufacturing & Supply Agreement]

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EXHIBIT A

DEFINITIONS

This **Exhibit A** to this Manufacturing and Supply Agreement provides agreed upon definitions applicable to the Parties for purposes of this Agreement. All capitalized terms used in this Agreement without definition shall have the meanings ascribed thereto in this **Exhibit A**.

1.1 Definitions.

"**Additional Raw Materials Cost**" means the costs of the Raw Materials associated with the re-Manufacture or Reprocessing of a Failed Batch or Defective Batch of Drug Product or Bulk Drug Substance under Section 8.6, which costs are comprised of (a) with respect to Drug Product, the cost of any additional Raw Materials necessary for the supply of any substitute Bulk Drug Substance that must be replaced therefor and the cost of any other additional Raw Materials necessary for Manufacture of the new Batch or to Reprocess the current Batch of Drug Product; and (b) with respect to Bulk Drug Substance, the cost for the supply of Raw Materials necessary to Manufacture a new Batch or Reprocess the current Batch of Bulk Drug Substance.

"**Affiliate**" means, with respect to any Person, any other Person directly or indirectly controlled by, controlling or under common control with such Person; and with respect to Catalent, it means [**], and any corporation, firm, partnership or other entity controlled by [**]. For purposes of this definition, "**control**" means the ownership of more than fifty percent (50%) of the securities or other ownership interests representing the equity voting stock or general partnership or membership interest of such entity or the power to direct or cause the direction of the management or policies of such entity whether through the ownership of voting securities, by contract or otherwise. The Parties acknowledge that in the case of certain entities organized under the Laws of certain countries outside of the U.S., the maximum percentage ownership permitted by Laws for a foreign investor may be less than fifty percent (50%), and that in such case, such lower percentage shall be substituted in the preceding sentence; provided, that such foreign investor has the power to direct or cause the direction of the management and policies of such Person; provided; further for purposes of this Agreement: (a) notwithstanding anything to the contrary, Sarepta and Catalent shall be deemed not to be an "Affiliate" of one another and (b) a Person shall cease to be an "Affiliate" hereunder upon the date that such Person no longer satisfies the requirements set forth in this definition.

"**Batch**" means a specific quantity of drug substance [**], Bulk Drug Substance or Drug Product, as applicable in the given context, that is intended to have uniform character and quality within specified limits and is produced according to a single cycle of Manufacture.

"**Batch Record**" means a manufacturing record for a Batch generated by Catalent concurrently with the production of a specific Batch such that successive steps in such processes are documented.

"**[**] Facility**" shall mean the product development and manufacturing facility located at [**].

"**BLA**" means a Biologics License Application (or successor or equivalent application) (including all supplements, amendments, and modifications thereof) for authorization for marketing of a biologic product, as defined in the applicable Laws and regulations and filed with applicable Regulatory Authorities, including an application for accelerated approval.

"**Bulk Drug Substance**" means the active pharmaceutical ingredients of the Lead DMD Product being Manufactured by Catalent in bulk form and prior to any formulation, filling, and finishing.

"**Business Day**" means a day other than a Saturday, Sunday, or other day on which commercial banks in New York are authorized or required by Law to be closed for business.

"**[**] Facility**" shall mean the commercial scale biomanufacturing facility located at [**].

"**[**] Facility**" shall mean the commercial scale biomanufacturing facility located at [**].

"**Catalent Facility**" and "**Catalent Facilities**" shall mean the [**] Facility, [**] Facility, and/or [**] Facility, individually or collectively as appropriate under the circumstances based upon the facility/facilities at which Manufacturing or other services are being performed.

"**Catalent Intellectual Property**" means any Intellectual Property owned or Controlled by Catalent prior to the Original Effective Date, or otherwise arising outside the performance of this Agreement, without reliance on or reference to any Sarepta Confidential Information.

"**Cell Line**" means the cell line licensed to or used by Sarepta for the Manufacture of the Lead DMD Product and any derivatives, modifications or progeny thereof.

"**Certificate of Analysis**" or "**COA**" means a written certificate issued by Catalent listing the items tested, the Specifications, testing methods and test results for a specific Batch.

"**Certificate of Compliance**" or "**COC**" means a written certificate issued by Catalent, in a form approved by the Parties and executed by Catalent's quality assurance department, certifying that each Batch has been Manufactured in accordance with and satisfies the Product Requirements.

"**cGCPs**" means the then-current standards, practices and procedures promulgated or endorsed by (i) the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use ("**ICH**") Harmonised Tripartite Guideline for Good Clinical Practice (CPMP/ICH/135/95) and any other guidelines for good clinical practice for trials on medicinal products in the EU, (ii) the FDA as set forth in the guidelines entitled "Guidance for Industry E6 Good Clinical Practice: Consolidated Guidance," including related regulatory requirements imposed by the FDA and (iii) the equivalent Laws in any relevant country, in each case, including all applicable rules, regulations, orders and guidance applicable thereto, and as each may be amended from time to time, and any successor thereto.

"**cGLPs**" means the then-current standards, practices and procedures promulgated or endorsed by (i) the European Commission Directive 2004/10/EC relating to the application of the principles of good laboratory practices as well as "The rules governing medicinal products in the European Union," Volume 3, Scientific guidelines for medicinal products for human use (ex – OECD principles of GLP), (ii) the then current good laboratory practice standards promulgated or endorsed by the FDA as defined in 21 C.F.R. Part 58, and (iii) the equivalent Laws in any relevant country, in each case, including all applicable rules, regulations, orders and guidance applicable thereto, and as each may be amended from time to time, and any successor thereto.

"**Change in Control**" means the occurrence of any of the following events:

(a) a sale, conveyance or other disposition of all or substantially all of the assets or lines of business of [**],

(b) a merger or consolidation with or into any other entity, unless the stockholders of [**] immediately before the transaction own fifty percent (50%) or more of the voting stock of the acquiring or surviving corporation following the transaction (taking into account, in the numerator, only shares of capital stock of [**] held by such stockholders before the transaction and capital stock issued in respect of such prior-held capital stock of [**]), or

(c) any other transaction which results in (assuming an immediate and maximum exercise or conversion of all derivative securities issued in the transaction) the holders of the capital stock of [**] as of immediately before the transaction owning less than fifty percent (50%) of the voting power of [**] capital stock as of immediately after the transaction.

"**Commercial Batch(es)**" means a Batch or Batches ordered in accordance with Section 5.2, in contrast to Pre-Launch Batch(es) ordered as part of the SOW.

"**Commercialize**," "**Commercializing**" or "**Commercialization**" means all activities directed to the marketing (whether through direct, in-person, electronic or other marketing channels), promotion, selling or offering for sale of a product for an indication, including planning, market research, pre-marketing activities undertaken in preparation for launch, advertising, educating, marketing, promoting, importing, exporting, distributing and post-marketing safety surveillance and reporting. For clarity, "Commercialize," "Commercializing" or "Commercialization" shall not include any activities included within the Manufacturing or Development of a product.

"**Competitive Product**" means, with respect to the Lead DMD Product, any therapeutic drug product [**].

"**Control**," "**Controls**" or "**Controlled**" means, when used in reference to intellectual property, other intangible property, or materials, that a Party owns or has a license or sublicense to such intellectual property, other intangible property or materials, and has the ability to grant a license or sublicense or other right to use such intellectual property, other intangible property or

materials, as applicable, as provided for herein, without (i) requiring the consent of a Third Party or (ii) violating the terms of any agreement or other arrangement with any Third Party.

"**Cover**," "**Covering**" or "**Covered**" means, with respect to a country in the Territory, but for a license granted under a valid claim of a Patent, the use or sale, or offer for sale in such country of the subject matter at issue would infringe such valid claim, or in the case of a Patent that is a Patent application, would infringe a valid claim in such Patent application if it were to issue as a Patent.

"**Defect**" means, with respect to a Batch, other than a Batch that is not intended for use in the manufacture of Drug Product intended for human use (such as a Batch produced in engineering run), a defect that causes the Batch to fail to conform to the Specifications or otherwise cannot be used due to the failure of the Batch to meet the Product Requirements, and which is (a) discovered by Sarepta upon review of the Batch Documentation or Actual Sarepta Receipt of the Batch or (b) a Latent Defect.

"**Develop**," "**Developing**" or "**Development**" means any and all activities relating to research, non-clinical, preclinical and clinical trials, toxicology testing, statistical analysis, publication and presentation of research and study results and reporting, process and analytical development, analytical testing, preparation and submission of applications (including any CMC-related information) for regulatory approval of a product, necessary or reasonably useful or otherwise requested or required by a Regulatory Authority as a condition or in support of obtaining or maintaining all Regulatory Approvals for such product. For clarity, "Development" shall not include any activities included within the Manufacturing of a product.

"**Drug Product**" means the formulated mixture of the Bulk Drug Substance and any excipients, finished and filled in final marketed dosage form.

"**EMA**" means the European Medicines Agency or its successor.

"**EU**" means the countries of the European Union as it exists at any time.

"**Exclusive Activities**" means performing or agreeing to perform any [**].

"**FD&C Act**" means the U.S. Federal Food, Drug and Cosmetic Act, as amended, and the regulations promulgated thereunder.

"**FDA**" means the U.S. Food and Drug Administration or its successor.

"**Fiscal Year**" means the calendar year commencing on January 1 and concluding on December 31.

"**GAAP**" means United States generally accepted accounting principles, as in effect from time to time, consistently applied.

"**Good Manufacturing Practices**" or "**cGMPs**" means the then-current good manufacturing practices required by (i) the FDA, as set forth in the FD&C Act and the regulations promulgated thereunder, for the manufacture and testing of pharmaceutical materials, including

the provisions of 21 C.F.R. Parts 210 and 211, (ii) European Commission Directive 91/356/EEC, as amended by Directive 2003/94/EC, and 91/412/EEC respectively, as well as "The rules governing medicinal products in the European Union," Volume 4, Guidelines for good manufacturing practices for medicinal products for human and veterinary use, and (iii) the principles detailed in the ICH Q7A guidelines, in each case, including all applicable rules, regulations, orders and guidance applicable thereto, and as each may be amended from time to time, and any successor thereto.

"**Governmental Authority**" means any multinational, federal, state, local, municipal or other governmental authority of any nature (including any governmental division, prefecture, subdivision, department, agency, bureau, branch, office, commission, council, court or other tribunal), in each case, having jurisdiction over the applicable subject matter.

"**Improvements**" means all discoveries, Inventions, developments, modifications, innovations, updates, enhancements or improvements to Intellectual Property, including to Sarepta Technology, (whether or not protectable under Patent, trademark, copyright or similar Laws) that are conceived, discovered, invented, developed, created, made or reduced to practice in the performance of the Parties' obligations under this Agreement.

"**IND**" means both the application of an Investigational New Drug Application to the FDA and its equivalent in other countries and their Regulatory Authorities, such as a clinical trial application or a clinical trial exemption, the filing of which is necessary to commence or conduct clinical testing of a pharmaceutical product in humans in such jurisdiction.

"**Invention**" means any subject matter invented during the Term, whether or not recorded or recognized as such during the Term, by or on behalf of a Party or one or more of the Parties jointly, as determined in accordance with the provisions of U.S. patent Law governing inventions, in the performance of the Parties' obligations under this Agreement.

"**Intellectual Property**" means all information, data, works of authorship, discoveries, concepts, methods, Know-How, designs, processes, software, algorithms and inventions, whether patentable or not, including, without limitation, those that could be the subject of patent, copyright, industrial design, trade secret or other forms of protection; including, without limitation, all (i) Patents; (ii) trademark applications, registrations, service marks, domain names and all renewals and extensions thereto; and (iii) copyright applications and registrations and all restorations, reversions, renewals and extensions thereof.

"**JSC**" has the meaning ascribed to it in the Amended and Restated Collaboration Agreement.

"**Know-How**" means any proprietary data, results, material(s), and nonpublic information of any type whatsoever, in any tangible or intangible form, including know-how, trade secrets, practices, techniques, methods, processes, inventions, discoveries, developments, specifications, formulations, formulae, materials or compositions of matter of any type or kind (patentable or otherwise), software, algorithms, marketing reports and plans, market research, expertise (including experts' information), test data (including pharmacological, biological, chemical,

biochemical, toxicological, preclinical and clinical test data), analytical and quality control data, stability data, other study data and procedures.

"**Latent Defect**" means a Defect that is not discoverable upon inspection and analysis of the Batch Documentation or upon inspection using commercially reasonable efforts at the time of Actual Sarepta Receipt but is discovered at a later time.

"**Laws**" means, with respect to Sarepta, all laws, statutes, rules, regulations, directives, decisions, ordinances, guidelines and other pronouncements of any Governmental Authority currently in effect or enacted or promulgated during the Term, and as amended from time to time, of each jurisdiction in which the product is produced, marketed, distributed, used or sold; and with respect to Catalent, all laws, statutes, rules, and regulations of any Governmental Authority currently in effect or enacted or promulgated during the Term, and as amended from time to time, of the jurisdiction in which Catalent Manufactures the Drug Product, including cGMP.

"**Lead DMD Product**" means Sarepta's lead microdystrophin gene therapy drug targeting Duchenne muscular dystrophy, which may also be referred to as SRP-9001.

"**Lead DMD Product Launch**" means the filing of a BLA or other commercial registration dossier for the Lead DMD Product using Catalent and Suites 3 and 4 as the manufacturer and site of Manufacture.

"**Lead DMD Product Scope of Work**" means the general terms of which are attached to this Agreement as **Exhibit B,** inclusive as **Exhibit B-1** (Process Development) and **Exhibit B-2** (Analytical Testing), relative to Technology Transfer Activities, Development Activities, the cGMP Manufacturing Runs, and the Process Performance Qualification, among others, which include proposed work stages for the performance of the technology transfer, engineering runs, and drug substance, drug product and analytical validation; timing of performance of the same; associated costs; and drug substance and drug product pricing.

"**Manufacture**" or "**Manufacturing**" means all activities, whether performed by a Party or a Third Party designee of a Party, related to the manufacturing of a product, or any ingredient thereof, including manufacturing for clinical use or commercial sale, formulation, filling and finishing activities, in process and product testing, release of product, quality assurance activities related to manufacturing and release of product, handling and storage of product and ongoing stability tests, packaging and labeling, and regulatory activities related to any of the foregoing.

"**Manufacture/Release Period**" means the period of time required for the Manufacture and release of a single Batch of Bulk Drug Substance of a Sarepta Drug Product, commencing on the date Catalent commences the Manufacture of such Batch and concluding on the submission of the corresponding Batch Documentation (which includes required release testing results) to Sarepta for its review, which period of time shall be determined by Catalent and affirmed by the JSC.

"**Manufacturing Configuration**" means the number of upstream production runs [**] and the subsequent downstream purification [**].

"**Marketing Authorization**" means an approval and authorization, including any renewals thereof, of the applicable Regulatory Authority necessary for the manufacture, packaging, marketing, storage, import, export, transport, distribution, sale and use of a pharmaceutical or biologic product in any country.

"**Marketing Authorization Application**" or "**MAA**" means an application to the appropriate Regulatory Authority for approval to sell a Drug Product (but excluding Pricing Approval) in any particular country or regulatory jurisdiction, including such application filed with the EMA pursuant to the Centralized Procedure or with the applicable Regulatory Authority of a country in accordance with such country's national approval procedure.

"**Material Testing Cost(s)**" means out of pocket costs incurred by Catalent to perform testing required by applicable Laws, a Scope of Work, or the Quality Agreement, as applicable, to determine or confirm the identity or characteristics of Raw Materials.

"**Out-of-Pocket Costs**" means costs and expenses paid to Third Parties (or payable to Third Parties and accrued in accordance with GAAP), other than Affiliates or employees of the Parties, by a Party.

"**Pass-Through Costs**" means Catalent's actual costs for [**] in each case, (a) through (i), for costs incurred for Manufacture of Bulk Drug Substance or Drug Product or the performance of services under a SOW.

"**Patent Term Extension**" means any term extensions, supplementary protection certificates, Regulatory Exclusivity and equivalents thereof offering Patent protection beyond the initial term with respect to any issued Patents.

"**Patents**" means (i) all national, regional and international patents and patent applications, including provisional patent applications, (ii) all patent applications filed either from such patents, patent applications or provisional applications or from an application claiming priority from either of these, including divisionals, continuations, continuations-in-part, provisionals, converted provisionals and continued prosecution applications, (iii) any and all patents that have issued or in the future issue from the foregoing patent applications ((i) and (ii)), including utility models, petty patents and design patents and certificates of invention, (iv) any and all extensions or restorations by existing or future extension or restoration mechanisms, including revalidations, reissues, re-examinations and extensions (including any supplementary protection certificates and the like) of the foregoing patents or patent applications ((i), (ii), and (iii)) and (v) any similar rights, including so-called pipeline protection or any importation, revalidation, confirmation or introduction patent or registration patent or patent of additions to any of such foregoing patent applications and patents.

"**Person**" means any corporation, limited or general partnership, limited liability company, joint venture, trust, unincorporated association, governmental body, authority, bureau or agency, any other entity or body, or an individual.

"**Pre-Launch Batch(es)**" means a Batch or Batches ordered as part of the SOW, in contrast to Commercial Batch(es) ordered in accordance with Section 5.3. For the avoidance of doubt, it might be possible to order Pre-Launch Batches following the launch of the Lead DMD Product,

for example, under an amendment to the SOW related to process optimization work outside the scope of routine Manufacturing.

"**Pre-Launch Period**" means the period starting on January 1, 2021 and ending on the date Sarepta receives Product Approval for the Lead DMD Product.

"**Post-Launch Period**" means the period beginning the day after Sarepta receives Product Approval for the Lead DMD Product and ending on the last day of the Term.

"**Pricing Approval**" means the approval, agreement, determination or decision from a Governmental Authority establishing the price and/or reimbursement for a Sarepta Drug Product for sale in a given country or regulatory jurisdiction, as required by applicable Laws in such country or other regulatory jurisdiction prior to the sale of the Sarepta Drug Product in such country or regulatory jurisdiction.

"**Procurement Fee**" means a fee applied to Pass-Through Costs, excluding insurance costs to be borne by Sarepta as described in Section 5.8, [**].

"**Product Approval**" means, with respect to a Drug Product, the approval of a Governmental Authority necessary for the marketing and sale in a given country or regulatory jurisdiction, which may include the approval of an MAA (but shall not include any Pricing Approvals).

"**Product Requirements**" shall mean, with respect to each Batch Manufactured pursuant to this Agreement, that such Batch shall: (a) conform to the applicable Specifications; (b) be manufactured, packaged, labeled, handled, and stored in compliance with applicable Laws and Regulatory Acts, including Regulatory Approvals, and the Quality Agreement; (c) contain only Raw Materials that have been used, handled or stored in accordance with the Storage Guidelines, applicable Laws and Regulatory Acts, and the Quality Agreement; and (d) not be adulterated or misbranded within the meaning of the United States Federal Food, Drug and Cosmetic Act, as amended ("**FDCA**"), or within the meaning of any applicable state or municipal law in which the definitions of adulteration or misbranding are substantially the same as those contained in the FDCA, as such Act and such law are constituted and effective at the time of delivery and will not be an article which may not, under provisions of Sections 404, 505 or 512 of the FDCA, be introduced in interstate commerce.

"**Project Manager**" means the representative(s) appointed by each Party and responsible for overseeing and coordinating the conduct of the Manufacturing and the completion of the Scope of Work by Catalent.

"**Purchase Order**" means a written or electronic order form submitted by Sarepta in accordance with the terms of this Agreement to Catalent authorizing the manufacture and supply of Bulk Drug Substance and/or Drug Product.

"**Quality Agreement**" means a detailed document specifying the quality and regulatory procedures and responsibilities of the Parties with respect to the Manufacture of the Lead DMD Product, entered into pursuant to Section 6.1 by and between Sarepta and Catalent. All references

to Quality Agreement herein shall refer to the Quality Agreement in effect at the time of the Manufacture of the Lead DMD Product.

"**Raw Materials**" means all physical materials to be consumed in the Manufacture of Bulk Drug Substance or Drug Product or incorporated into Bulk Drug Substance or Drug Product or the packaging thereof, including process consumables, packaging materials, and components needed for the Manufacture of Bulk Drug Substance and/or Drug Product.

"**Readiness Determination**" has the meaning ascribed to it in the Amended and Restated Collaboration Agreement.

"**Regulatory Acts**" means any rules, regulations, directives, decisions, ordinances, guidelines and other pronouncements, including Product Approvals, of any Regulatory Authority currently in effect or enacted or promulgated during the Term, and as amended from time to time.

"**Regulatory Approvals**" means, with respect to a Drug Product or a facility for the Manufacture of a Drug Product or component thereof, all filings and approvals (including, as applicable, IND filings, Product Approvals, Pricing Approvals, establishment license approvals and, in each case any supplements and amendments thereto), licenses, registrations or authorizations of any Governmental Authority necessary to obtain Marketing Authorization for or to Develop, Manufacture or Commercialize a Drug Product, as applicable, for or in a country or regulatory jurisdiction.

"**Regulatory Authority**" means any Governmental Authority involved in granting Regulatory Approval for or in a country or regulatory jurisdiction in which the product is (or in which Sarepta, its Affiliates or sublicensees is applying for authority for it to be) produced, marketed, distributed, used or sold, including [**].

"**Regulatory Exclusivity**" means, with respect to Drug Products, any exclusive marketing rights or data exclusivity rights conferred by any Governmental Authority with respect to the Drug Products other than a Patent right, including in the European Union, Regulation (EC) No 726/2004 and Directive 2001/83/EC (as amended).

"**Regulatory Materials**" means, with respect to Drug Products or the facilities used to Manufacture Drug Products or a component thereof, as applicable, regulatory applications, submissions, notifications, communications, correspondence, registrations, Regulatory Approvals and/or other filings made to, received from or otherwise conducted with a Regulatory Authority that are necessary in order to obtain marketing authorization for or to Develop, Manufacture or Commercialize a Drug Product or to use a facility for the Manufacture thereof, for or in a country or regulatory jurisdiction, including all rights under the foregoing, including rights to clinical data and Regulatory [**]. Regulatory Materials include BLAs, INDs, MAAs, pre-registration documents, registration documents, presentations, responses, applications for Product Approvals and granted Product Approvals.

"**Regulatory Warning Notices**" means Form FDA 483 Inspectional Observations, Establishment Inspection Reports, warning letters, or their equivalents and any similar

correspondences received from the FDA or any other Governmental Authority having jurisdiction over a Drug Product or any facility for the Manufacture of a Drug Product or component thereof.

"**Representatives**" means a Party's (and its Affiliates') directors, officers, full-time employees, part-time employees, temporary workers, subcontractors, consultants, agents, permitted sublicensees (if any) and legal, technical, and business advisors.

"**Reprocess**" or "**Reprocessing**" means introducing a Batch back into, and repeating appropriate manipulation steps that are part of, the established Manufacturing process.

"**Sarepta Clean Room Suites**" means two (2) clean room suites at the [**] Facility referred to as Suites 3 and 4 [**] that will be dedicated to Sarepta for Manufacturing of Sarepta Drug Products.

"**Sarepta Intellectual Property"** means any Intellectual Property, including Sarepta Patents, owned or Controlled by Sarepta prior to the Original Effective Date, or otherwise arising outside the performance of this Agreement, without reliance on or reference to any Catalent Confidential Information.

"**Sarepta Materials**" means any or all of the master cell bank, working cell bank and/or research cell bank vials, the Cell Line, and the DNA plasmids, as provided by or made available to Catalent by or on behalf of Sarepta or its predecessor in interest, or as Developed by Catalent for purposes of Manufacturing the Lead DMD Product, as the same may be amplified, processed or improved by Catalent in accordance with this Agreement, as well as all information provided by or on behalf of Sarepta concurrently therewith that specifically relates thereto.

"**Sarepta Patents**" means any Patent that is owned or Controlled by Sarepta as of the Original Effective Date or comes under the ownership or Control of Sarepta during the Term and (i) is necessary for or useful to the Development, Manufacture, use or Commercialization of Sarepta Drug Products or (ii) relates to any Sarepta Drug Products. For purposes of this definition, Sarepta shall not be deemed to Control any Patent that is licensed by Catalent to Sarepta pursuant to this Agreement.

"**Sarepta Technology**" means (i) any present and future Intellectual Property that is owned or Controlled by Sarepta and necessary for or useful to the Development, Manufacture or Commercialization of Sarepta Drug Products and (ii) any Intellectual Property that is developed or obtained by or on behalf of Sarepta related to Sarepta Drug Products, or the Manufacture of any of the foregoing, (a) prior to the Original Effective Date, or (b) independent of this Agreement and without reliance on or reference to the Confidential Information of Catalent. Sarepta Technology specifically includes any Sarepta Materials. For purposes of this definition, Sarepta Technology shall not include any Intellectual Property that is licensed by Catalent to Sarepta pursuant to this Agreement. For clarity, Sarepta Technology includes any Development or Manufacturing Know-How originating outside of this Agreement and from any of Sarepta's other contract manufacturers in relation to any Sarepta Drug Product.

"**Specifications**" shall mean, with respect to the Bulk Drug Substance or Drug Product, all specifications for Raw Materials, approved suppliers, formula, Manufacturing, analytical and

testing procedures, Yield, release, packaging, storage, labeling, artwork and other processes relating to the Manufacture of Bulk Drug Substance or Drug Product, as determined by Sarepta and provided to Catalent, including all master formulas, process flow diagrams, all packaging and filling work orders and all specifications and requirements contained in the BLA, all if and as amended from time to time by the Parties, and all requirements that may be included in the applicable regulatory files for the Lead DMD Product as it relates to the Bulk Drug Substance and Drug Product.

"**Storage Costs**" means the out of pocket costs incurred by Catalent for storage at a Third Party storage facility, which shall be mutually agreed to by the Parties on an annual basis. The first agreed upon annual amount shall be [**]. For the avoidance of doubt, the term "Storage Costs" excludes costs associated [**].

"**Storage Guidelines**" means those procedures, methods and conditions for preserving, monitoring and storing all Sarepta Materials, Raw Materials, Bulk Drug Substance and/or Drug Product, as set forth in the Quality Agreement or as otherwise mutually agreed to in writing by the Parties.

"**Strategic Partners**" means the individuals or entities that are part of a strategic partnership, which is a relationship between two commercial enterprises formalized by one or more business contracts.

"**Sublicensee**" means any Third Party to which Sarepta, its Affiliates or Strategic Partners, or any of its or their sublicensees grants any or all of the rights licensed by Catalent to Sarepta under this Agreement.

"**Territory**" means [**]. Catalent shall not be obliged to process products for sale in any of such countries if it is prevented from doing so, or would be required to obtain or apply for special permission to do so, due to any restriction (such as an embargo) imposed on it by any governmental authority, including those imposed by the U.S. Department of the Treasury's Office of Foreign Assets Control.

"**Third Party**" means any Person other than the Parties.

"**U.S.**" means the United States of America and its possessions and territories.

"**Yield**" means the total viral genome produced per Batch of Bulk Drug Substance.

1.2 Additional Definitions. The following terms have the meanings set forth in the corresponding Sections of this Agreement:

Defined Term	**Section**
Acquisition	Recitals
Action	18.3(c)
Actual Sarepta Receipt	8.2(b)
Additional Batches	5.3(e)
Administered Rules	20.2(b)

Agreement Preamble
Amended and Restated Collaboration Agreement Recitals
Approved Production Schedule 7.4(a)
Arising IP Patents 15.8
Auditing Party 11.9
Audit 17.2(b)
Batch Documentation 9.2(d)
Batch Investigation 7.1(c)
Batch Materials Cost 5.8
Batch Price 5.5
Batch Processing 7.1(a)
Binding Orders Period 5.3(c)
Binding Quarter or BQ 5.3(c)
BOM 5.8
Catalent Preamble
Catalent Arising IP 15.4
Catalent Indemnitee 18.7
Catalent Records 17.1(a)
Catalent Financial Records 17.2(a)
cGMP Manufacturing Run 4.1
Chemistry Manufacturing and Control or CMC 11.1
CIP Savings 10.5(b)
Confidential Information 14.1
Continuous Improvement Program 10.1(h)
CPS Recitals
CPR 20.2(b)
Dedicated Clean Room Collaboration Recitals
Default 13.2
Defective Batch 7.1(b)
Delay Period 7.4(a)
Delivery 5.9(b)
Deposit 5.8
Deposit Invoice 5.8
Development Activities 4.1
Disclosing Party 14.1(a)
Effective Date Preamble
Estimate 4.7(c)(ii)
Excess Batch 5.3(a)
Extraordinary Increase 5.5(e)
Failed Batch 7.1(b)
Forecast Modification 5.3(e)
Force Majeure Event 13.1
Framework 7.1(d)
FY 5.5(e)
Indemnified Party 18.8
Indemnifying Party 18.8

Initial Forecast 5.3(c)(i)
Initial Forecast Refresh 5.3(c)(ii)
Key Performance Indicators 5.4
Lead DMD Product Technology Transfer 5.15
Loss 18.6
Maximum Annual Batches 5.3(a)
Maximum Upstream Runs 5.3(a)
Minimum Annual Threshold 5.2(b)
Minimum Order Quantity 5.2(b)
Non-Binding Quarter 5.3(d)
Non-BOM Materials 5.8
Original Collaboration Agreement Recitals
Original Effective Date Recitals
Original Lead DMD Agreement Recitals
Payment Schedule 4.7(a)
PPI 5.5(e)
PPQ Completion Date 4.4
PPQ Lots 4.4
Process Development-Focused SOWs Recitals
Process Performance Qualification or PPQ 4.1
Production Schedule 5.3(f)
Receiving Party 14.1(a)
Retained Copies 19.2(j)
Rolling Forecast 5.3(d)
Root Cause 7.1(c)
Rules 20.2(b)
Sarepta Preamble
Sarepta Approval 4.6(a)
Sarepta Arising IP 15.5
[**] 8.6(d)
[**] 8.6(d)
Sarepta Drug Products Recitals
Sarepta Indemnitee 18.6
Scope of Work 4.1
Target 7.1(d)
Technology Transfer Activities 4.1
Technology Transfer Fee 5.15(c)
Technology Transfer License 5.15(a)
Term 19.1(a)
Terminating Event 7.1(a)
Termination Date 19.2(a)
Transferees 5.15
[**] 19.2(c)
[**] 8.6(d)
[**] 19.2(b)

DOCPROPERTY DOCXDOCID DMS=InterwovenIManage Format=<<NUM>>_<<VER>> PRESERVELOCATION * MERGEFORMAT 137289020_3

EXHIBIT B

Lead DMD Product Scope of Work

DOCPROPERTY DOCXDOCID DMS=InterwovenIManage Format=<<NUM>>_<<VER>> PRESERVELOCATION * MERGEFORMAT 137289020_3

EXHIBIT B-1

Process Development Scope of Work

Exhibit B-1-1

DOCPROPERTY DOCXDOCID DMS=InterwovenIManage Format=<<NUM>>_<<VER>> PRESERVELOCATION * MERGEFORMAT 137289020_3

EXHIBIT B-2

Analytical Testing Scope of Work

DOCPROPERTY DOCXDOCID DMS=InterwovenIManage Format=<<NUM>>_<<VER>> PRESERVELOCATION * MERGEFORMAT 137289020_3

EXHIBIT C

Approved Subcontractors

[**]

Exhibit C-1

DOCPROPERTY DOCXDOCID DMS=InterwovenIManage Format=<<NUM>>_<<VER>> PRESERVELOCATION * MERGEFORMAT 137289020_3

EXHIBIT D

Sarepta Therapeutics: Notice of Batch Forecasts

Sarepta Therapeutics [**] Rolling Forecast of Bulk Drug Substance and Drug Product

[**]

Exhibit D-1

DOCPROPERTY DOCXDOCID DMS=InterwovenIManage Format=<<NUM>>_<<VER>> PRESERVELOCATION * MERGEFORMAT 137289020_3

[**] = Identified information has been excluded from this exhibit because it is both (i) information that the Company customarily and actually treats as private or confidential and (ii) is not material.

Exhibit 10.2

[**] = IDENTIFIED INFORMATION HAS BEEN EXCLUDED FROM THIS EXHIBIT BECAUSE IT IS BOTH (I) INFORMATION THAT THE COMPANY CUSTOMARILY AND ACTUALLY TREATS AS PRIVATE OR CONFIDENTIAL AND (II) IS NOT MATERIAL

EXCLUSIVE LICENSE AGREEMENT
BETWEEN
THE RESEARCH INSTITUTE AT NATIONWIDE CHILDREN'S HOSPITAL
AND
SAREPTA THERAPEUTICS, INC.

This Exclusive License Agreement (the "Agreement") is entered into as of the last date of the signatures below (the "Effective Date") by and between the Research Institute at Nationwide Children's Hospital, 700 Children's Drive, Columbus, OH 43205 ("Research Institute") and Sarepta Therapeutics, Inc., a Delaware corporation having offices at 215 First Street, Cambridge, MA 02142 ("Licensee").

RECITALS

1. Research Institute has conducted and continues to conduct a research and clinical development program to advance the development of a microdystrophin-based gene therapy known as rAAVrh74.MHCK7.micro-dystrophin for the treatment of Duchenne muscular dystrophy, which program has resulted in certain intellectual property and data, and regulatory authorizations, including the Licensed Technology (as defined below).

2. Licensee is a biopharmaceutical company developing innovative RNA-targeted therapeutics for life threatening diseases including neuromuscular disorders.

3. Research Institute and Licensee are parties to an Exclusive Option Agreement dated December 29, 2016 (the "Exclusive Option Agreement") and a Sponsored Research Agreement dated December 29, 2016, (as amended, by the First Amendment thereto (the "First Amendment") dated as of April 1, 2018, the Second Amendment thereto (the "Second Amendment") dated as of April 29, 2018, and the Third Amendment thereto (the "Third Amendment") dated as of August 30, 2018, the "Sponsored Research Agreement"), pursuant to which agreements Licensee has the option to acquire a license under the Institution Project IP and under the Option Technology. Pursuant to the Sponsored Research, the safety and efficacy of a microdystrophin-based gene therapy known as rAAVrh74.MHCK7.micro-dystrophin currently is being evaluated in a Phase I/IIa clinical trial (ClinicalTrials.gov Identifier: NCT03375164) (the "Phase I/IIa Trial").

4. Research Institute and Licensee are parties to the following agreements in support of Licensee's rights under the Exclusive Option Agreement and its participation in the Sponsored Research: (i) a Non-Exclusive License Agreement (the "Non-Exclusive License Agreement") dated as of April 19, 2018; (ii) a Material Transfer Agreement (the "Transgene MTA") dated as of April 27, 2018; (iii) a Material Transfer Agreement (the

"Tissue Sample MTA") dated as of April 25, 2018; (iv) a Technology Transfer and Non-Exclusive License Agreement (the "Analytical Method Technology Transfer Agreement") dated as of April 18, 2018; and (v) a Technology Transfer, Consultancy and Non-Exclusive License Agreement (the "Production Process Technology Transfer Agreement") dated as of April 18, 2018.

5. Licensee has exercised the Option and the Sponsor Option, and Research Institute is willing to grant Licensee a license to exploit the Licensed Technology as required by the Exclusive Option Agreement and the Sponsored Research Agreement in accordance with the terms and conditions set forth below.

In consideration of the mutual promises contained herein and other good and valuable consideration, the receipt and sufficiency of which is hereby agreed, Research Institute and Licensee hereby agree as follows:

ARTICLE I
DEFINITIONS AND INTERPRETATION

1.1 Definitions. The capitalized terms used herein shall have the meanings set forth below in this Section 1.1 unless otherwise expressly defined in this Agreement.

1.1.1 "Affiliate" shall mean any entity that, at the relevant time, directly or indirectly owns or controls, is owned or controlled by, or is under common ownership or control with a Party to this Agreement. For the purpose of this definition, "ownership" or "control" shall mean: (a) the direct or indirect possession or ownership of greater than fifty percent (50%) of the outstanding voting stock of the entity; (b) the right to receive more than fifty percent (50%) of the profits or earnings of the entity; (c) the power to appoint or remove a majority of the board of directors of the entity; or (d) the power to direct the management and policies of the entity. For clarity, an entity's status as an Affiliate shall terminate if, at any time, such entity is not within the definition listed above; provided; however, the obligations of that entity shall continue until their purposes are fulfilled in accordance with the terms and conditions of this Agreement.

1.1.2 "Affiliate Defined Under the SRA" shall mean with respect to any person or entity, any other person or entity that directly or indirectly controls, is controlled by or is under common control with such person or entity, during the term of such control. A person or entity will be deemed to be "controlled" by any other person or entity if such other person or entity: (a) possesses, directly or indirectly, power to direct or cause the direction of the management and policies of such person or entity whether by contract or otherwise, (b) has direct or indirect ownership of fifty percent (50%) or more (in the aggregate) of the voting power of all outstanding shares entitled to vote at a general election of directors of the person or entity or (c) has direct or indirect ownership of fifty percent (50%) or more of the equity interests in a partnership or a limited liability company.

1.1.3 "Ancillary Agreements" shall mean collectively the Non-Exclusive License Agreement, the Transgene MTA, the Tissue Sample MTA, the Analytical Method Technology Transfer Agreement, and the Production Process Technology Transfer Agreement.

1.1.4 "Change of Control" shall mean: (a) the acquisition, either directly or indirectly, by any Third Party of more than fifty percent (50%) of the voting stock of Licensee; (b) any merger or consolidation, including a series of transactions amounting to the foregoing, involving Licensee that requires a vote of the stockholders of Licensee; or (c) the transfer to any Third Party of all or substantially all the assets of Licensee relating to the subject matter of this Agreement.

1.1.5 "Commercially Reasonable Efforts" shall mean, with respect to a Licensed Product, efforts and resources that are no less than those committed by other companies in the pharmaceutical, biopharmaceutical or biotechnology industry for the research, development or commercialization of similarly situated products or services at a similar stage of development or in a similar stage of product life, with similar developmental risk profiles, of similar market and commercial potential, taking into account relevant factors, including the competitiveness of the market place, the proprietary position of the products or services and that of third parties, patient availability, and the regulatory structure involved. Commercially Reasonable Efforts requires that Licensee: (a) promptly assign responsibility for such obligations or tasks to specific employee(s) who are held accountable for progress and monitor such progress on an on-going basis; (b) set and consistently seek to achieve specific and meaningful objectives for carrying out such obligations; and (c) consistently make and implement decisions and allocate resources designed to advance progress with respect to such objectives.

1.1.6 "Designated Manufacturer" shall mean Brammer Bio MA, LLC or another entity approved in writing by Research Institute.

1.1.7 "EMA" shall mean the European Medicines Agency, or any successor agency thereof.

1.1.8 "End User" shall mean a Third Party (natural or otherwise) whose use of a Licensed Product results in its consumption, destruction, loss of activity and/or loss of value.

1.1.9 "Exclusive Licensed Patents" shall mean all Patent Rights, Institution Patent Rights, Joint Patent Rights, and Mixed-Product Patent Rights. Exhibit A-1 sets forth a list of the Exclusive Licensed Patents existing as of the Effective Date in accordance with the following categories: (i) Patent Rights, (ii) Institution Patent Rights, (iii) Joint Patent Rights, and (iv) Mixed-Product Patent Rights.

1.1.10 "Exclusive Technical Information" shall mean any Technical Information that: (a) is or relates solely to product specifications, chemical structure, process descriptions, analytical testing reports, clinical protocols, regulatory correspondence and filings, or preclinical data, including preclinical pharmacology, drug metabolism and pharmacokinetics data, for a Licensed Product; (b) is described in Exhibit E; and (c) is not in the public domain as a result of activities by Research Institute; and does not include the Technical Information listed in Exhibit F.

1.1.11 "FDA" shall mean the United States Food and Drug Administration, or any successor agency thereof.

1.1.12 "Field of Use" shall mean all uses of Licensed Products for Duchenne Muscular Dystrophy and Becker Muscular Dystrophy in humans.

1.1.13 "First Commercial Sale" shall mean the first use, sale, transfer or other disposition for value of a Licensed Product that is not a Non-Commercial Disposition by Licensee or its Sublicensees after such Licensed Product has received Regulatory Approval.

1.1.14 "Gene Therapy Product" shall mean the gene therapy product that is: (a) the subject of the Sponsored Research, including the product known as [**].

1.1.15 "Institution" shall mean Research Institution.

1.1.16 "Institution Inventions" shall mean all Inventions conceived, made, created, developed or, in the case of any patentable Invention, Invented, during the Term as Defined in the SRA, in the planning or conduct of the Sponsored Research, solely by one or more employees of Institution.

1.1.17 "Institution Patent Rights" shall mean any and all: (a) patent applications, including all provisional applications, priority applications, continuations, continuations-in-part, divisions and all patents, reissues and reexaminations granted thereon; (b) all supplementary protection certificates and extensions thereof; and (c) all U.S. and foreign counterparts of any of the foregoing; in each case claiming Institution Inventions.

1.1.18 "Institution Project IP" shall mean Institution Inventions, Institution Patent Rights and Institution's interest in the Joint Inventions and Joint Patent Rights.

1.1.19 "Invented" shall mean the act of invention by inventors, as determined in accordance with United States patent laws.

1.1.20 "Invention" shall mean any discovery, invention, creation, improvement or modification, whether or not patentable, including, but not limited to, processes, methods, formulas, technical information, materials, compositions, formulas, biological materials, assays, compounds,

techniques, computer software and documentation, data and know-how, together with any patent, copyright or other intellectual property rights therein.

1.1.21 "Joint Inventions" shall mean all Inventions conceived, made, created, developed or, in the case of any patentable Invention, Invented, during the Term as Defined in the SRA, in the planning or conduct of the Sponsored Research, jointly by one or more employees of Licensee or its Affiliates Defined Under the SRA and one or more employees of Institution.

1.1.22 "Joint Patent Rights" shall mean any and all: (a) patent applications, including all provisional applications, priority applications, continuations, continuations-in-part, divisions and all patents, reissues and reexaminations granted thereon; (b) all supplementary protection certificates and extensions thereof; and (c) all U.S. and foreign counterparts of any of the foregoing; in each case claiming Joint Inventions.

1.1.23 "License Year" shall mean each calendar year during the Term, provided that the first License Year shall begin on the Effective Date of this Agreement and run until December 31 of the same calendar year and the final License Year shall end on the date of expiration or termination of this Agreement.

1.1.24 "Licensed Inventions" shall mean: (a) the Institution Inventions and Research Institute's entire rights, title and interests in and to Joint Inventions for which the applicable Sponsor Option has been exercised; and (b) the Technology; in each case excluding [**].

1.1.25 "Licensed Patents" shall mean collectively Exclusive Licensed Patents, Platform Patents and Other Patents.

1.1.26 "Licensed Product" shall mean each of: (a) the Gene Therapy Product; and (b) any gene therapy product that: [**].

1.1.27 "Licensed Technology" shall mean the Licensed Patents, the Licensed Inventions and the Technical Information.

1.1.28 "Licensed Territory" shall mean worldwide.

1.1.29 "Materials" shall mean all materials identified on Exhibit C of the Sponsored Research Agreement.

1.1.30 "Milestone Event" shall mean any milestone event set forth in Section 4.2.2 corresponding to a Milestone Payment.

1.1.31 "Milestone Payment" shall mean any milestone payment set forth in Section 4.2.2 corresponding to a Milestone Event.

1.1.32 "Mixed-Product Patent Rights" shall mean: (a) all patents or patent applications owned or controlled by Research Institute that claim the Gene Therapy Product: (i) identified in Exhibit A-1 as Mixed-Product Patent Rights; or (ii) disclosed to Research Institute during the Term as Defined in the SRA; (b) all patents and patent applications filed in any country in the Licensed Territory that directly or indirectly claim priority to, have common priority with, or claim the benefit of, the patents or patent applications described in clause (a), including without limitation all provisional or priority patent applications, divisionals, continuations, continuations-in-part, reissues, reexaminations, supplementary protection certificates, international applications and utility models; (c) all patents granting from (a) and (b); and (d) all extensions based on any of the foregoing; in each case only to the extent claiming the Gene Therapy Product.

1.1.33 "NCH Technology" shall have the meaning set forth in Exhibit F, incorporated herein by reference.

1.1.34 "Net Sales" shall mean the gross amount invoiced (and any amounts received but not invoiced) by Licensee or any of its Affiliates or other Sublicensees for the use, sale, transfer or other disposition for value of Licensed Products to the End User, less the following, to the extent documented as attributable to the Licensed Products: [**]. In the event Licensed Products are put into use, sold, transferred or otherwise disposed of other than in an arms-length transaction, except for a Non-Commercial Disposition, or if Licensee or its Affiliates or other Sublicensees receive consideration other than cash for Licensed Products, then the invoiced amount shall be [**]. Notwithstanding the foregoing, [**].

1.1.35 "Non-Commercial Disposition" shall mean the use, sale, transfer or other disposition of a Licensed Product by or among Licensee, its Sublicensees and a Third Party as follows: (a) any transfer of a Licensed Product by Licensee or its Sublicensees for a bona fide charitable purpose without consideration of any kind, including a compassionate use program, patient assistance program, named patient, expanded access or other similar programs, so called "treatment IND sales," "named patient sales" or use under the ATU system in France or other equivalent systems; (b) any transfer of a Licensed Product for use in studies where the Licensed Product is provided by Licensee or its Sublicensees without charge, or a commercially reasonable number of units of Licensed Product transferred for no consideration for marketing purposes (e.g., samples), but not for resale by the Third Party.

1.1.36 "Option" shall mean an exclusive option to acquire a license under the Option Technology in the Territory for all uses in humans.

1.1.37 "Option Product" shall mean: (a) any product or process, including a service, claimed by a Valid Claim or whose use is claimed by a Valid Claim;

(b) [**]; and (c) any other product or process with respect to which Licensee, or an Affiliate or Sublicensee, incorporates or references some or all of the Exclusive Technical Information in its new drug application or marketing authorization application submitted to the FDA or the EMA to seek approval to market such product in the United States or in the European Union; in each case: (i) excluding [**]; and (ii) only to the extent of the gene therapy product expressing micro-dystrophin and not to the extent of any other protein expressed.

1.1.38 "Option Product IND" shall mean any investigational new drug application relating to an Option Product that Research Institute files with the FDA or any foreign equivalent thereof, including the Product IND.

1.1.39 "Option Technology" shall mean the Patent Rights, the Technology, and the Option Product IND.

1.1.40 "Option Term" shall mean the period beginning on December 29, 2016 and ending: (a) two (2) months following the date of delivery by Research Institute to Licensee of: (i) pre-treatment dystrophin expression data and (ii) the first post-treatment dystrophin expression data, in each of (i) and (ii) obtained from all patients, including any placebo patients, under the Phase I Clinical Study or Phase II Clinical Study of the first Option Product to undergo such a Phase I Clinical Study or Phase II Clinical Study pursuant to the Research Plan in the Sponsored Research Agreement; or (b) the Effective Date.

1.1.41 "Orphan Drug Designation" shall mean a designation under Section 526 of the Federal Food, Drug, and Cosmetic Act as amended by section 2 of the Orphan Drug Act (sections 525-528 (21 U.S.C. 360aa-360dd)) or any grant of a corresponding designation by a corresponding Regulatory Authority outside of the United States.

1.1.42 "Other Patents" shall mean: (a) all patents or patent applications owned or controlled by Research Institute identified in Exhibit A-2; (b) all patent and patent applications filed in any country in the Territory that directly or indirectly claim priority to, have common priority with, or claim the benefit of, the patents or patent applications described in clause (a), including without limitation all provisional or priority patent applications, divisionals, continuations, continuations-in-part, reissues, reexaminations, supplementary protection certificates, international applications and utility models; (c) all patents granting from (a) and (b); and (d) all extensions based on any of the foregoing; in each case only to the extent claiming the Gene Therapy Product. Exhibit A-2 sets forth a list of the Other Patents existing as of the Effective Date.

1.1.43 "Party" shall mean either Licensee or Research Institute, and "Parties" shall mean both Licensee and Research Institute.

1.1.44 "Patent Rights" shall mean: (a) all patents or patent applications owned or controlled by Research Institute claiming the Technology; (b) all patent and patent applications filed in any country in the Territory that directly or indirectly claim priority to, have common priority with, or claim the benefit of, the patents or patent applications described in clause (a), including without limitation all provisional or priority patent applications, divisionals, continuations, continuations-in-part, reissues, reexaminations, supplementary protection certificates, international applications and utility models; (c) all patents granting from (a) and (b); and (d) all extensions based on any of the foregoing.

1.1.45 "Phase I Clinical Trial" shall mean an initial study of an investigational new drug in humans designed to determine the metabolism and pharmacological actions of the drug in humans and the side effects associated with increasing doses, as described in 21 C.F.R. §312.21(a) or the equivalent regulation in a foreign jurisdiction.

1.1.46 "Phase I/II Clinical Trial" shall mean an initial study of an investigational new drug in humans that is designed to satisfy the requirements of 21 C.F.R. § 312.21(a) and (b) or the equivalent regulation in a foreign jurisdiction.

1.1.47 "Phase II Clinical Trial" shall mean a study of an investigational new drug in humans to determine the safety, dose ranging or efficacy of the drug in humans, as described in 21 C.F.R. §312.21(b) or the equivalent regulation in a foreign jurisdiction.

1.1.48 "Phase III Clinical Trial" shall mean a study of an investigational new drug in humans that is designed to satisfy the requirements of 21 C.F.R. § 312.21(c) or the equivalent regulation in a foreign jurisdiction.

1.1.49 "Platform Patent Rights" shall mean: (a) all patents or patent applications owned or controlled by Research Institute that claim [**]; (b) all patents and patent applications filed in any country in the Licensed Territory that directly or indirectly claim priority to, have common priority with, or claim the benefit of, the patents or patent applications described in clause (a), including without limitation all provisional or priority patent applications, divisionals, continuations, continuations-in-part, reissues, reexaminations, supplementary protection certificates, international applications and utility models; (c) all patents granting from (a) and (b); and (d) all extensions based on any of the foregoing; in each case only to the extent claiming the Gene Therapy Product.

1.1.50 "Product IND" shall mean the complete investigational new drug application that Research Institute files with the FDA with respect to the Gene Therapy Product with the IND number identified on Exhibit E.

1.1.51 "Regulatory Approval" shall mean with respect to a particular Licensed Product, receipt of all regulatory clearances, registrations, licenses, authorizations or approvals (which in the case of the E.U. may be through the centralized procedure) required in the jurisdiction in question for the sale of the applicable product or service in such jurisdiction, including receipt of pricing approval, if any, legally required for such sale.

1.1.52 "Regulatory Authority" shall mean any applicable government regulatory health authority involved in granting clearances or approvals for the manufacturing, marketing or pricing of a Licensed Product, including the FDA and the EMA.

1.1.53 "Regulatory Exclusivity" shall mean, with respect to a Licensed Product in a country, any exclusive marketing right, data exclusivity right or other exclusivity status conferred by any Regulatory Authority with respect to such Licensed Product in such country, other than an Exclusive Licensed Patent, that limits or prohibits a Third Party from: (a) relying on pivotal safety or efficacy data generated by or for the Parties with respect to a Licensed Product in an application for Regulatory Approval of a product or (b) commercializing a Licensed Product.

1.1.54 "Research Personnel" shall mean [**].

1.1.55 "Research Plan" shall mean the research plan set forth in Exhibit A of the Sponsored Research Agreement.

1.1.56 "Research Results" shall mean all data and information generated in the performance of the Sponsored Research and any research reports furnished to Licensee under the Sponsored Research Agreement, including descriptions of experiments conducted under the Research Plan and corresponding analyses and conclusions, but excluding Institution Inventions, Sponsor Inventions and Joint Inventions.

1.1.57 "Royalty Period" shall mean on a country-by-country and Licensed Product-by-Licensed Product basis, the period commencing on the First Commercial Sale of a Licensed Product in a country and ending upon the later of: (a) the expiration of the last to expire Valid Claim of the Exclusive Licensed Patents in such country, or (b) the end of Regulatory Exclusivity of such Licensed Product in such country.

1.1.58 "Royalty Quarter" shall mean each of the calendar quarter periods during the Term following the First Commercial Sale of a Licensed Product; provided that the first Royalty Quarter of this Agreement shall begin on the date of the First Commercial Sale of a Licensed Product and end on the last day of the calendar quarter in which such date occurs.

1.1.59 "Sponsor" shall mean Licensee.

1.1.60 "Sponsor Inventions" shall mean all Inventions conceived, made, created, developed or, in the case of any patentable Invention, Invented, during the Term as Defined in the SRA, in the planning or conduct of the Sponsored Research, solely by one or more employees or independent contractors of Sponsor or its Affiliates Defined Under the SRA.

1.1.61 "Sponsor Option" shall mean an exclusive option to acquire an exclusive, world-wide, sublicensable license under the Institution Project IP pursuant to the Sponsored Research Agreement.

1.1.62 "Sponsored Research" shall mean the activities described in the Research Plan.

1.1.63 "Sublicense" shall mean any agreement, however captioned and regardless of how the conveyances are referred to therein, in which Licensee directly or indirectly: (a) grants or otherwise conveys any of the rights licensed hereunder; (b) agrees not to assert any of the rights licensed hereunder; (c) has agreed not to practice any right licensed hereunder, regardless of how Licensee refers to such person or entity therein; and/or (d) permit the making, offering for sale using, selling or importing of Licensed Product. For clarity, a permitted assignment of this Agreement in accordance with Section 12.2 hereof shall not be deemed a Sublicense hereunder.

1.1.64 "Sublicense Remuneration" shall have the meaning set forth in Section 4.5.

1.1.65 "Sublicensee" shall mean any entity granted rights directly or indirectly through a Sublicense.

1.1.66 "Technical Information" shall mean in each case to the extent selected by Research Institute to provide hereunder and existing as of the Effective Date, or during the Term as Defined in the SRA to the extent arising thereunder: (a) research and development data and information, unpatented inventions, and know-how pertaining to specific Licensed Products, including manufacturing information and any other data or information generated prior to the Effective Date; and (b) tangible materials pertaining to or comprising specific Licensed Products; in each case that is reasonably necessary or useful for the research, development, manufacture or commercialization of such specific Licensed Products; provided, however, manufacturing information is limited to that known as of the Effective Date. For clarity, Technical Information shall include all information and tangible materials described in Exhibit F but solely to the extent that it relates to the Gene Therapy Product defined in Section 1.1.14(a).

1.1.67 "Technology" shall mean the inventions described in [**].

1.1.68 "Term as Defined in the SRA" shall mean the term, unless earlier terminated in accordance with the terms of the Sponsored Research Agreement,

beginning on the Effective Date (as defined in the Sponsored Research Agreement) and ending upon the earlier of (a) the date of completion of all activities relating to the Sponsored Research, as set forth in the Research Plan or b) the date that is [**] after the Effective Date (as defined in the Sponsored Research Agreement).

1.1.69 "Territory" shall mean all countries in the world.

1.1.70 "Third Party" shall mean any person or entity other than the Parties.

1.1.71 "Valid Claim" shall mean: (i) a claim of an issued patent included within the Exclusive Licensed Patents, which claim has not (a) expired, lapsed, been permanently canceled, dedicated to the public, disclaimed or become abandoned, (b) been declared unpatentable, invalid, unenforceable, revoked, or canceled by a final decision or judgment of a court or other appropriate body or authority of competent jurisdiction that is not appealable or cannot be appealed, or (c) been admitted to be invalid or unenforceable; or (ii) a claim of a pending application included within the Exclusive Licensed Patents, which claim has not been canceled, withdrawn, abandoned or disallowed without the possibility of appeal or re-filing of the application and that has not been continuously pending for more than [**] from the earliest priority date from which such claim takes priority, unless and until such claim becomes an issued claim of an issued patent in which case it shall again be considered a Valid Claim under clause (i) above.

1.2 Interpretation. Each definition in this Agreement includes the singular and the plural. References to any statute or regulation mean such statute or regulation, as amended from time to time, and include any successor legislation, regulations, guidelines and policies promulgated therefrom. The headings to the Articles and Sections are for convenience of reference and shall not affect the meaning or interpretation of this Agreement. The Exhibits attached hereto are hereby incorporated by reference into and shall be deemed a part of this Agreement. The term "including" shall mean "including but not limited to."

ARTICLE II
LICENSE GRANTS AND RESERVATION OF RIGHTS

2.1 Grants.

2.1.1 Patent and Invention Licenses. Subject to the terms and conditions of this Agreement and Licensee's compliance therewith, Research Institute hereby grants to Licensee an exclusive, royalty-bearing, non-transferable (except as provided under Section 12.2) license in the Field of Use and Licensed Territory, with the right to grant Sublicenses (as provided in Section 2.1.3), under (i) all Exclusive Licensed Patents, and (ii) all Licensed Inventions, in each of (i) and (ii), solely to make, have made, use, sell, offer to sell, and import Licensed Products throughout the Licensed Territory solely within the Field of Use. For clarity, subject to Research Institute's reserved rights

under Section 2.3 and compliance with Section 2.4, the foregoing license is exclusive even as to Research Institute.

2.1.2 Technical Information License. Subject to the terms and conditions of this Agreement and Licensee's compliance therewith, Research Institute hereby grants to Licensee a non-transferable (except as provided under Section 12.2) license in the Field of Use and Licensed Territory, with the right to grant Sublicenses (as provided in Section 2.1.3), to use the Technical Information solely to research, develop, manufacture and commercialize Licensed Products throughout the Licensed Territory and for only the Field of Use. The foregoing license is exclusive as to the Exclusive Technical Information and non-exclusive as to any Technical Information that is not Exclusive Technical Information. The Technical Information is provided "AS IS." Research Institute shall transfer a copy of or give access to all Exclusive Technical Information, solely for a Licensed Product in the Field of Use, as set forth on Exhibit E in its possession or control to Licensee within thirty (30) days of the Effective Date unless otherwise set forth on Exhibit E or already provided under the Ancillary Agreements, and otherwise have no further obligation regarding the Technical Information. Subject to Section 13.1, the Technical Information is provided to Licensee solely for the purpose as set forth in this Section 2.1.2 and no other purpose, with no equitable or legal title transferring. Exclusive Technical Information shall be returned or its destruction certified upon request by Research Institute upon the termination of this Agreement, subject to Section 11.3.1. Nothing herein shall be construed as a sale of the Technical Information. Licensee is obtaining access to the Licensed Technology solely for a Licensed Product in the Field of Use, but not the secrecy thereof. Research Institute is not obligated to maintain the Technical Information, excluding the Exclusive Technical Information solely for a Licensed Product in the Field of Use, as Confidential Information; provided, however, that the terms and conditions of Section 10.4 shall apply to Technical Information solely for a Licensed Product in the Field of Use.

2.1.3 Covenant Not to Sue. Subject to the terms and conditions of this Agreement and Licensee's compliance therewith, Research Institute, for itself, its Affiliates and its or their respective heirs, executors, administrators, successors, and assigns, hereby covenants and irrevocably agrees that it shall not, anywhere in the Licensed Territory, directly or indirectly assert any legal or equitable cause of action, suit or claim against Licensee or its Sublicensees (the "Covenant Beneficiaries") asserting infringement or other violation of the Other Patents and the Platform Patents in the Field of Use solely for the making, having made, using, selling, offering for sale or importation of any Gene Therapy Product in the Licensed Territory within the Field of Use. The foregoing right under this Section 2.1.3 is non-transferable (except as provided under Section 12.2).

2.1.4 Sublicenses. Subject to the terms and conditions of this Agreement and Licensee and each Sublicensee's compliance therewith, Licensee may grant Sublicenses through multiple tiers to the rights licensed to Licensee in Sections 2.1.1 and 2.1.2 provided that Licensee does so by written agreement consistent with the terms and conditions of this Agreement. Licensee agrees to provide Research Institute with: (a) the identity of any Sublicensee; and (b) a true, correct and complete copy of the terms of any Sublicense and any amendment to it; provided that, Licensee may redact from such copy confidential terms of such Sublicense that relate to the technical characteristics of any product or otherwise are not necessary for Research Institute to monitor compliance by Licensee or such Sublicense with the terms and conditions of this Agreement. Each Sublicense shall terminate upon termination of this Agreement; provided that Research Institute shall enter into licenses with Sublicensees pursuant to Section 11.3.2. Licensee shall remain liable for each Sublicensee's compliance with the Sublicense as if such entity was performing as Licensee under the terms and conditions of this Agreement, and Research Institute shall have the right to audit Sublicensees to ensure such compliance, subject to Section 4.7. Notwithstanding anything to the contrary herein, only a Designated Manufacturer may be a Sublicensee of the NCH Technology.

2.1.5 Additional Designated Manufacturer. Technology transfer to additional Designated Manufacturers may be made in a subsequent technology transfer and consultancy agreement having substantially the same terms as the Production Process Technology Transfer Agreement.

2.1.6 Affiliates. Research Institute acknowledges and accepts that subject to the terms and conditions of this Agreement and Licensee's and its Affiliate's compliance therewith, Licensee may exercise its rights and perform its obligations under this Agreement either directly or through one or more of its Affiliates acting as a Sublicensee, under a Sublicense in compliance with Section 2.1.3. Without limiting other terms and conditions of this Agreement Licensee will remain responsible for the acts and omissions, including financial liabilities, of its Affiliates as if such Affiliate was performing as Licensee under the terms and conditions of this Agreement.

2.2 No Encumbrances. Except as otherwise permitted under Section 2.2, Research Institute shall not enter into any agreement that grants any Third Party any right or license, or option or other right to obtain any right or license, under any Licensed Technology to the extent of the Licensed Product in the Field of Use (other than the Other Patents, Platform Patents or Technical Information that is not Exclusive Technical Information). Licensee acknowledges and agrees that Research Institute is permitted to practice and have practiced, including without limitation to grant any Third Party any right or license under, intellectual and/or tangible property rights including without limitation the Licensed Technology, to express oligonucleotides that when provided to humans lead to the expression of dystrophin.

2.3 Reservation of Rights.

2.3.1 Research Institute reserves on behalf of itself and its Affiliates: (a) all rights, titles and interests not expressly granted in Sections 2.1.1 and 2.1.2; (b) the right to practice and have practiced the Licensed Technology for the purpose of performing non-profit, non-clinical research for internal educational and teaching purposes (and the right to publish the results of such non-clinical research, subject to Article X); and (c) the right to use the Licensed Technology to perform the Sponsored Research. For purposes of clarity, notwithstanding anything to the contrary in this Agreement, the terms of the Sponsored Research Agreement shall control with respect to the publication of any clinical trial data or results that are Research Results.

2.3.2 This Agreement does not convey and Research Institute retains all rights, titles and interests, including conveyances by implication, estoppel or otherwise, in tangible or intangible property rights, including any patents, know-how, tangible materials, or other inventions or discoveries, that are not the Licensed Technology except as granted in Sections 2.1.1 and 2.1.2.

2.4 Government Rights. Licensee understands that the Licensed Patents may have been conceived or may be first actually reduced to practice with funding from the U.S. government. Licensee agrees that (a) all rights granted under the Licensed Patents shall be limited by and subject to the rights of the U.S. government, as applicable, and Licensee shall comply and enable Research Institute to comply with all obligations to the U.S. government, including those set forth in 35 U.S.C. §200 et al., regarding substantially manufacturing and practicing Licensed Products covered by such Licensed Patents in the U.S., unless waived; and (b) on an annual basis, Licensee shall report to Research Institute whether or not it qualifies as a "small business firm" as defined in 37 C.F.R. 401.14(a)(5).

2.5 Acknowledgement. Licensee, on behalf of itself, its Affiliates and Sublicensees, including the Designated Manufacturer, agrees not to practice or have practiced the Licensed Technology outside the Field of Use.

ARTICLE III
DUE DILIGENCE BY LICENSEE

3.1 Due Diligence by Licensee. Licensee represents and warrants to Research Institute, to induce Research Institute to enter into this Agreement, that Licensee: (a) shall use Commercially Reasonable Efforts to, either itself or through its Affiliates or other Sublicensees, develop and obtain Regulatory Approval to sell the Licensed Products, and make Licensed Products commercially available to the public within the Field of Use in any country in the Licensed Territory where Regulatory Approval has been obtained so that public utilization and practical application of the Licensed Technology results therefrom; and (b) has, or shall obtain within a reasonable period of time after the Effective Date, the expertise necessary to develop, market and sell Licensed Products.

3.2 Development Plan. Licensee has provided Research Institute with the development plan attached as Exhibit B describing the steps Licensee will take to develop the Licensed Technology and make or have made and sell Licensed Products in the Field of Use and throughout the Licensed

Territory; provided, however, Licensee shall be entitled, from time to time, to make such commercially reasonable adjustments to such development plan as Licensee believes, in its good faith judgment, are advisable, and consistent with Licensee's obligations in this Article 3.

3.3 Development Report. Within thirty (30) days following the end of each License Year, Licensee shall provide Research Institute with a written development report containing at least the information set forth in Exhibit C, and describing in detail: (a) as of that License Year, all development and marketing activities for each Licensed Product and the names of all Sublicensees, including Affiliates, to which Licensee has extended the license granted herein during such License Year; and (b) an updated development plan for the next License Year which shall, notwithstanding Section 12.3, amend Exhibit B of this Agreement when accepted by Research Institute.

3.4 Development Milestones. Licensee (either itself or through its Sublicensees) shall achieve the following development milestones by the dates specified, in each case with respect to one (1) Licensed Product. Licensee shall promptly notify Research Institute upon the achievement of each of the development milestones, identify whether the Licensee or its Sublicensee is responsible for the achievement of such milestone, and the actual date of such achievement. In the event that marketing of a Licensed Product commences in the United States following Regulatory Approval, all of the milestones set forth in Section 3.4 that have not previously been achieved will be deemed to have been achieved for the purposes of this Agreement. For example, if BLA approval of a Licensed Product is obtained on the basis of an accelerated approval without Phase III Clinical Trial data, then the milestone set forth in Section 3.4.3 shall be deemed to have been achieved.

3.4.1 [**]

3.4.2 [**]

3.4.3 [**]

3.4.4 [**]

3.5 Requirements. If prior to receiving BLA approval from the FDA and commencing marketing of a Licensed Product in the United States, Licensee believes that it will not achieve one or more of the Milestone Events specified in Section 3.4 by the deadlines indicated therein, it shall notify Research Institute in writing thereof in advance of the relevant deadline. Licensee shall include with such notice a reasonable explanation of the reasons for such failure despite having used Commercially Reasonable Efforts. If Licensee so notifies Research Institute and such explanation is acceptable to Research Institute (in its reasonable discretion), or, in any event, if such failure to meet the Milestone Event is due to circumstances beyond Licensee's reasonable control (such as patent infringement issues or regulatory issues), then the Parties shall negotiate in good faith a reasonable plan, including a reasonable extended timeframe, for achieving such milestone and upon agreement on the plan, and notwithstanding anything to the contrary herein, Licensee shall not be deemed to be in breach of the relevant milestone. In addition, if Licensee is in material breach of Section 3.4, the Research Institute may terminate this Agreement pursuant to Section 11.2.2. For clarity, if Licensee ceases development of a Licensed Product prior to achieving all the milestones in Section 3.4, but has commenced a Phase I Clinical Trial of a second Licensed

Product and continues with that Licensed Product to achieve the remaining milestones, then it will not be deemed to have breached Section 3.4 with respect to the first Licensed Product.

3.6 Remedy. Licensee's material failure to perform any of its obligations specified in Article 3 shall constitute a material breach of this Agreement and Research Institute shall have the right and option at its sole election to terminate this Agreement as provided in Section 11.2.2 (including its notice and cure requirements) in whole or in part, or convert Licensee's exclusive license to a non-exclusive license.

3.7 Development Records. Licensee shall continuously maintain customary documentation evidencing that Licensee is pursuing development of Licensed Products as required herein. Such documentation may include invoices for studies of Licensed Products, laboratory notebooks, internal job cost records, and filings made to the Internal Revenue Service to obtain tax credits, if available, for research and development of Licensed Products. Licensee shall permit Research Institute and/or an independent auditor who is reasonably acceptable to Licensee to audit the development records at Research Institute's expense subject to the same procedures and restrictions set forth for audit of financial records in Section 4.7.

ARTICLE IV
PAYMENTS

4.1 License Issue Fee. Licensee shall pay Research Institute a non-creditable and non-refundable license issue fee in the amount of [**] ($[**]), within seven (7) days of Effective Date. For the convenience of Licensee, Research Institute will provide Licensee with an invoice for the license issue fee. The license issue fee accrues and is due to Research Institute regardless of whether Research Institute issues any invoice.

4.2 License Maintenance Fee. Licensee shall pay Research Institute the non-creditable and non-refundable license maintenance fees and Milestone Payments as follows.

4.2.1 Annual Payment. Licensee shall pay to Research Institute [**] dollars ($[**]) per year, payable within [**] following each anniversary of the Effective Date; provided, however, that Licensee's obligation to pay this fee shall end on the date of the First Commercial Sale for which Licensee pays Research Institute the corresponding Annual Minimum pursuant to Section 4.4. For the convenience of Licensee, Research Institute will provide Licensee with an invoice for the annual payment. The annual payment accrues and is due to Research Institute regardless of whether Research Institute issues any invoice.

4.2.2 Milestone Payments.

Licensee shall pay to Research Institute Milestone Payments in the amounts specified below upon the first occurrence of each of the following Milestone Events for each Licensed Product to achieve such Milestone Event. Each Milestone Payment set forth in this Section 4.2.2 is payable only once per Licensed Product. For clarity, Licensee shall pay Research Institute no more

than [**] dollars ($[**]) in development Milestone Payments per Licensed Product and no more than [**] dollars ($[**]) in sales Milestone Payments per Licensed Product. Licensee shall notify Research Institute in writing within [**] following the achievement of each Milestone Event described in this Section 4.2.2. For the convenience of Licensee, Research Institute will provide Licensee with an invoice following receipt of such written notice from Licensee, and Licensee shall pay the associated Milestone Payment within [**] of the receipt of such invoice. These Milestone Payments accrue and are due to Research Institute regardless of whether Research Institute issues any invoice.

Development Milestone Event	*Milestone Payment*
[**]	$[**]
[**]	$[**]
[**]	$[**]
[**]	$[**]
[**]	$[**]
Sales Milestone Event	*Milestone Payment*
[**]	$[**]
[**]	$[**]
[**]	$[**]

Notwithstanding anything to the contrary herein, in the event that more than one of the Sales Milestone Events in this Section 4.2.2 is achieved in the same calendar year for the same Licensed Product and indication, [**].

4.3 Earned Royalty.

4.3.1 Licensee shall pay to Research Institute during the Royalty Period a non-creditable and non-refundable royalty of [**] percent ([**]%) of Net Sales of all Licensed Products.

4.3.2 Net Sales shall accrue upon the first of invoice, shipment or other disposition of Licensed Product.

4.3.3 In the event Licensee challenges the validity or enforceability of any of the Licensed Patents and is unsuccessful, [**].

4.4 Minimum Royalties. Commencing with the first full License Year that commences after the First Commercial Sale of a Licensed Product, Licensee shall pay Research Institute in each License Year a non-refundable annual minimum royalty payment in the amount of $[**] per License Year (the "Annual Minimum") within [**] following the first day of each License Year. The Annual

Minimum for a given License Year shall be creditable against the royalty payments made by Licensee to Research Institute under Section 4.3 in such License Year.

4.5 Sublicensing Payments. Licensee shall pay to Research Institute a percentage of the remuneration received by Licensee for each Sublicense, [**] (such remuneration, "Sublicense Remuneration"), as set forth below:

4.5.1 [**] percent ([**]%) of Sublicense Remuneration attributable to a Licensed Product received between the Effective Date and the completion of a Phase II Clinical Trial for such Licensed Product.

4.5.2 [**] percent ([**]%) of Sublicense Remuneration attributable to a Licensed Product received between the completion of a Phase II Clinical Trial for such Licensed Product and the first Regulatory Approval of such Licensed Product.

4.5.3 [**] percent ([**]%) of Sublicense Remuneration attributable to a Licensed Product received following the first Regulatory Approval of such Licensed Product.

For clarity, Sublicense Remuneration shall exclude [**]. Sublicensing payments shall be made to Research Institute by or on the due date of the report for the Royalty Quarter in which the Sublicense Remuneration was received or, if prior to First Commercial Sale, within [**] of receipt by Licensee.

4.6 Royalty Payment and Report. Licensee shall notify Research Institute of the achievement of the First Commercial Sale within [**] thereof. Within [**] after the end of each Royalty Quarter, Licensee shall provide to Research Institute a written report, due even if there are no Net Sales, detailing Licensee's and its Sublicensees' sales and development activities during the Royalty Quarter. Each report shall: (a) be substantially in the form attached as Exhibit D; (b) be certified as accurate and complete by an authorized official of Licensee or its Sublicensee; and (c) set forth a full accounting of any amounts due, including the description and number of Licensed Products manufactured, used, transferred and/or otherwise disposed of, the calculation of Net Sales of such Licensed Products on a country-by-country basis, including an itemized listing of any allowable deductions or credits, if any, under this Agreement, including Licensed Products used, sold, transferred or otherwise disposed of based on Non-Commercial Disposition under subsections (b) or (c) of the definition, the total royalty payment and remuneration due during such Royalty Quarter, any amounts due for Annual Minimums or milestones, exchange rates used and the method of calculation of amounts due Research Institute for such Royalty Quarter, including any sublicensing payments and royalties received and payable. Concurrent with the making of each such report, Licensee shall include payment due. If no payment is due for the Royalty Quarter, Licensee shall so state.

4.7 Accounting. Licensee shall keep and maintain and shall require all of its Sublicensees to keep and maintain complete, accurate, and continuous records for a period of six (6) years from the date of creation, which show the manufacture, transfer, use, and other disposition of Licensed Products. Such records shall include general ledger records showing cash receipts and expenses,

and records which include production records, customers, and related information, in sufficient detail to determine the amounts payable hereunder. Licensee shall permit Research Institute and/or an independent accountant who is reasonably acceptable to Licensee reasonable access once during any calendar year, to audit during ordinary business hours, such records as may be necessary to verify or determine royalties or other payments paid or payable under this Agreement; provided that Research Institute shall give Licensee reasonable prior written notice [**] prior to conducting any such audit, and that Research Institute or the independent accountant may audit any given period only once. Licensee may require the auditor to sign a customary nondisclosure agreement prior to undertaking any such inspection, and any and all books, records, reports and other documents inspected by such auditor shall be deemed Licensee's Confidential Information. The auditor shall not disclose to Research Institute any information other than the information relating to the accuracy of reports and payments delivered under this Agreement, and any such information delivered to Research Institute, including in the form of an audit report, shall be deemed Confidential Information of both Parties. Licensee shall pay Research Institute unpaid amounts due hereunder, plus interest as set forth in Section 4.9, within thirty (30) days after receiving a written audit report. Research Institute shall pay the cost and expense of the audit unless the results of the audit reveal an under-reporting or an underpayment due Research Institute of [**] percent ([**]%) or more, in which case Licensee shall reimburse Research Institute for the costs and expenses of the audit within [**] of receipt of invoice.

4.8 Self-audit. Licensee shall conduct [**] an independent audit at least every two (2) years if annual sales of Licensed Products are over [**] dollars ($[**]). The audit shall address, at a minimum, the amount of Net Sales during the audit period, any payment amounts owed to Research Institute under this Agreement, and whether the amounts owed have been paid to Research Institute and are reflected in the reports and records of the Licensee as reported to Research Institute. Licensee shall submit promptly to Research Institute: (a) the auditor's certified report upon completion; and (b) unpaid amounts due hereunder, plus interest as set forth in Section 4.9, within [**] after receiving such audit report.

4.9 Interest. The royalty and other payments set forth in this Agreement shall, if overdue, bear interest until payment at the monthly rate of [**] The acceptance of the payment of such interest shall not foreclose Research Institute from exercising any other rights or remedies it may have.

4.10 Payment Procedures. All payments due from Licensee hereunder shall be made in U.S. dollars by check or money order payable to the "Research Institute at Nationwide Children's Hospital." With respect to transfers in countries outside the United States, payments shall be made in U.S. dollars at the rate of exchange published in The Wall Street Journal on the close of business on the last banking day of each Royalty Quarter in which the royalty accrues. Such payments shall reference the Research Institute tax identification number [**] and shall be remitted to the address for Research Institute specified in Section 12.1 of this Agreement.

4.11 Taxes. All amounts payable to Research Institute under this Agreement are net of all taxes and other charges, and Licensee shall pay, and shall indemnify and hold the Research Institute harmless against, all taxes, transfer fees and other charges levied by any taxing authority on account of license fees, royalties or any other sums payable under this Agreement. Licensee shall deliver to Research Institute copies of all official tax receipts.

ARTICLE V
PATENT MANAGEMENT

5.1 Prosecution, Defense and Maintenance of Licensed Patents. Provided that Licensee timely makes all of its payments under this Agreement, Research Institute shall use reasonable efforts consistent with its normal practices to prosecute and maintain the Licensed Patents in the Field of Use and Licensed Territory and Licensee shall cooperate with all lawful requests of Research Institute in effectuating such efforts.

5.1.1 Consultation. Research Institute shall reasonably consult with Licensee on material matters regarding the prosecution of the Exclusive Licensed Patents within the Field of Use and Licensed Territory and implement all reasonable requests made by Licensee regarding such matters. Research Institute shall provide Licensee with a draft of all significant correspondence or filings with governmental patent offices related to the Exclusive Licensed Patents to review and comment in advance as reasonably possible in advance of filing and Research Institute shall cause any reasonable comments of Licensee to be included in any such correspondence or filing. Licensee shall make information available to Research Institute that is reasonably requested to support the prosecution of the Exclusive Licensed Patents.

5.1.2 Control. Without limiting its obligations to Licensee under Section 5.1.1, Research Institute has the right to make final decisions regarding Licensed Patents including determining whether or not, and where, to: (a) file and prosecute patent applications; (b) maintain the patents with respect to Licensed Patents; and (c) unless the Parties agree otherwise with respect solely to the Exclusive Licensed Patents, to defend Challenge Actions. If Licensee elects to discontinue payment for the Licensed Patents as otherwise required by Section 5.2.1, and provided such payments do not relate to any action or proceeding commenced at the request of Licensee, including without limitation a Challenge Action or Infringement Action, then Licensee shall give prompt written notice of such election to Research Institute at least [**] before the date of the corresponding patent application-related or patent-related action or fee is due, and in any event so as to provide Research Institute a reasonable amount of time to meet any applicable deadline to establish or preserve such patent or patent application in the applicable country or region if it elects to do so in its sole discretion. In the event that Licensee does not provide at least [**] notice, Licensee shall remain responsible for the documented costs and expenses incurred by Research Institute.

Notwithstanding anything to the contrary herein, Licensee is not permitted to discontinue payment for any action or proceeding commenced or defended at the request of Licensee, including without limitation a Challenge Action or Infringement Action. Failure to strictly comply with Licensee's payment obligations constitutes a material breach of this

Agreement for which Research Institute may terminate this Agreement pursuant to Section 11.2.2, without limiting any other rights or remedies available to Research Institute, including without limitation as provided under Section 5.2.2.

The Parties agree that they share a common legal interest to get valid enforceable patents and that each Party will maintain as privileged all information received from the other Party pursuant to this Section 5.1. The Parties agree and acknowledge that they have not waived, and nothing in this Agreement constitutes a waiver of, any legal privilege concerning the Licensed Patents or their Confidential Information, including privilege under the common interest doctrine and similar or related doctrines.

5.1.3 Notice. Each Party shall promptly inform the other Party of all matters that come to its attention that may affect the filing, prosecution, defense or maintenance of the Exclusive Licensed Patents in the Field of Use.

5.2 Patent Costs.

5.2.1 Costs. [**]

5.2.2 Loss of Rights, Continuing Payment Obligation. If Licensee has provided notice of its election to discontinue payment for the Licensed Patents as set forth in Section 5.1.2, or fails to pay any invoice submitted by Research Institute for those costs or expenses within [**]s after the date of that invoice, then the corresponding patent or patent application shall be excluded from the Licensed Patents, and all rights relating to such patent applications and patents included in or derived therefrom shall revert to Research Institute without further obligation to Licensee and may be freely licensed by Research Institute to others. If Licensee elects not to pay the patent costs for the filing, prosecution, defense and/or maintenance of any patent application or patent in any country, and Research Institute acting in reliance on that election ceases to prosecute that patent application, defend such rights or maintain that patent in that country where Research Institute has a good faith belief it was entitled to continue to prosecute, defend or maintain such patent or patent application, then Licensee agrees that it and its Sublicensees shall not sell any product or practice any process claimed in that patent as issued, or in the case of an application, claimed at the time Licensee notifies Research Institute of its decision not to support the application, unless Licensee pays royalties under this Agreement on sales in that country at the rate set forth in Section 4.3 as if such patent or patent application was included in the Licensed Patents.

5.3 Patent Term Extensions. For each Licensed Product, the Parties shall cooperate in: (a) selecting a patent within the Exclusive Licensed Patents to seek a term extension for; and/or (b) seeking a supplementary protection certificate in relation thereto from the Exclusive Licensed

Patents; each in accordance with the applicable laws of any country. Research Institute agrees to execute any documents and to take any additional actions as Licensee may reasonably request in connection therewith. [**].

5.4 Challenge. In the event Licensee challenges the patentability, validity or enforceability of any of the Licensed Patents, Licensee agrees that it shall: (a) give Research Institute [**] prior written notice; (b) continue to make all payments set forth herein directly to Research Institute; (c) not make any payment in escrow or other account; and (d) continue to comply and require any Sublicensee to comply with the terms and conditions of this Agreement. For purposes of clarity, no payment made to Research Institute is refundable or may be offset, including any amounts paid under this Agreement prior to or during the period of the challenge, even if the challenge is successful or it is otherwise determined that the Licensed Patents do not include valid claims.

ARTICLE VI
INFRINGEMENT

6.1 Notice. Each Party shall promptly notify the other Party in writing of any actual or suspected infringement of any Exclusive Licensed Patent by a Third Party in the Field of Use regarding a product that what would be Licensed Product if licensed hereunder of which it becomes aware and furnish all available evidence thereof ("Infringement Notice"). Each Party shall promptly notify the other Party in writing of its receipt of a notification of a biosimilar application based on a Licensed Product for which a biosimilar application is filed with the FDA by a Third Party pursuant to 42 U.S.C. §262(k). Both Parties shall use reasonable efforts and cooperate to terminate infringement in the Licensed Territory and within the Fields of Use without litigation.

6.2 Licensee Abatement.

6.2.1 Commencing on the Effective Date, [**]. In furtherance of such right, Research Institute shall cooperate with Licensee, including considering in good faith joining an Infringement Action as reasonably requested (except that Research Institute shall not be required to join suit even if joinder is required for Licensee to maintain standing due to procedural requirements), and executing all applicable documents necessary for Licensee to initiate, and prosecute such actions or proceedings, without expense to Research Institute. [**].

As used herein, any action or proceeding (including a declaratory judgment action, interference, opposition, *inter partes* review, or other post-grant proceeding or nullification action) brought in a court of law or a patent office that challenges the patentability, validity or enforceability of any Licensed Patent, requires the defense of the Licensed Patents or that seeks a determination that any Infringing Product does not infringe or misappropriate any Licensed Patent is a "Challenge Action".

6.3 Research Institute Abatement.

6.3.1 If, within [**] after the date of the Infringement Notice, [**].

6.4 Settlement. The abating Party shall have the right to reasonably settle an action filed pursuant to Section 6.2 or Section 6.3, provided that such settlement does not: (i) impose any material obligations on the other Party beyond the obligations imposed on either Party under this Agreement; (ii) compromise the Exclusive Licensed Patents; or (iii) admit fault. In the event the abating Party is Licensee, the foregoing does not permit Licensee to grant rights other than to a Sublicensee pursuant to Section 2.1.4.

ARTICLE VII
REPRESENTATIONS, WARRANTIES, COVENANTS AND DISCLAIMERS

7.1 Licensee's Representations and Warranties. Licensee represents and warrants to Research Institute that: (a) it is and shall be at all times during the Term a valid legal entity existing under the law of its state with the power to own all of its properties and assets and to carry on its business as it is currently being conducted; (b) the execution and delivery of this Agreement has been duly authorized and no further approval, corporate or otherwise, is required in order to execute this valid, binding and enforceable Agreement; (c) it shall comply with the terms and conditions of this Agreement and all applicable international, national, or local laws and regulations in its performance under this Agreement and development, manufacture and sale, use, transfer and other disposition of the Licensed Products; and (d) its execution, delivery, and performance of this Agreement shall not conflict in any material fashion with the terms of any other agreement or instrument to which it is or becomes a party or by which it is or becomes bound.

7.2 Research Institute's Representations and Warranties. Research Institute represents and warrants to Licensee that: (a) it is and shall be at all times during the Term a valid legal entity existing under the law of its state with the power to own all of its properties and assets and to carry on its business as it is currently being conducted; (b) the execution and delivery of this Agreement has been duly authorized and no further approval, corporate or otherwise, is required in order to execute this valid, binding and enforceable Agreement; (c) it has the right to grant the licenses granted hereunder; and (d) to Research Institute's knowledge without an obligation to investigate, its execution, delivery and performance of this Agreement shall not conflict with the terms of any other agreement to which it is a party or by which it is bound.

7.3 Disclaimers. NEITHER PARTY MAKES ANY REPRESENTATION OR WARRANTY NOT EXPRESSLY SET FORTH IN THIS AGREEMENT AND EACH PARTY ON BEHALF OF ITSELF AND ITS AFFILIATES AND SUBSIDIARIES EXPRESSLY DISCLAIMS ALL REPRESENTATIONS AND WARRANTIES WHETHER EXPRESS, STATUTORY, IMPLIED OR OTHERWISE, INCLUDING MERCHANTABILITY AND FITNESS FOR A PARTICULAR PURPOSE, ARISING FROM ANY COURSE OF DEALING, USAGE, OR TRADE PRACTICE, WITH RESPECT TO THE SCOPE, VALIDITY OR ENFORCEABILITY OF THE LICENSED TECHNOLOGY; THAT ANY PATENT SHALL ISSUE BASED UPON ANY OF THE PENDING LICENSED PATENTS; REGARDING THE TECHNICAL INFORMATION, INCLUDING THE EXCLUSIVE TECHNICAL INFORMATION; OR THAT THE MANUFACTURE, USE, SALE, OFFER FOR SALE OR IMPORTATION OF LICENSED PRODUCTS SHALL NOT INFRINGE INTELLECTUAL PROPERTY RIGHTS. THE ENTIRE RISK AS TO PERFORMANCE OF LICENSED PRODUCTS IS ASSUMED BY LICENSEE. LICENSEE AGREES THAT IN NO EVENT SHALL THE Research Institute, ITS AFFILIATES, SUBSIDIARIES AND THEIR RESPECTIVE OFFICERS, DIRECTORS, EMPLOYEES,

REPRESENTATIVES, STUDENTS, INDEPENDENT CONTRACTORS OR AGENTS, BE RESPONSIBLE OR LIABLE FOR ANY INDIRECT, SPECIAL, INCIDENTAL, CONSEQUENTIAL, PUNITIVE OR OTHER DAMAGES WHATSOEVER, WHETHER GROUNDED IN TORT (INCLUDING NEGLIGENCE AND PRODUCT LIABILITY), STRICT LIABILITY, CONTRACT OR OTHERWISE. NOTWITHSTANDING THE FOREGOING, NOTHING SHALL LIMIT Research Institute REMEDIES OR ABILITY TO RECOVER DAMAGES, INCLUDING INCREASED DAMAGES, FOR WILLFUL INFRINGEMENT OR MISAPPROPRIATION IN THE EVENT Research Institute ASSERTS ITS INTELLECTUAL PROPERTY RIGHTS. Research Institute AGREES THAT IN NO EVENT OTHER THAN PURSUANT TO SECTION 8.1 SHALL LICENSEE, ITS AFFILIATES, SUBSIDIARIES AND THEIR RESPECTIVE OFFICERS, DIRECTORS, EMPLOYEES, REPRESENTATIVES, STUDENTS, INDEPENDENT CONTRACTORS OR AGENTS, BE RESPONSIBLE OR LIABLE FOR ANY INDIRECT, SPECIAL, INCIDENTAL, CONSEQUENTIAL OR PUNITIVE DAMAGES. THE ABOVE LIMITATIONS ON LIABILITY APPLY EVEN IF ADVISED OF THE POSSIBILITY OF SUCH DAMAGE.

7.4 No Warranties to Third Parties. Licensee shall not make any statements, representations or warranties or accept any liabilities or responsibilities whatsoever that are inconsistent with this Agreement.

ARTICLE VIII
INDEMNITY & INSURANCE

8.1 Indemnity. Licensee on behalf of itself and its Affiliates, Sublicensees and subcontractors shall indemnify, hold harmless, and defend Research Institute, its Affiliates and subsidiaries, and their respective officers, directors, employees, representatives, students, agents, and independent contractors ("Research Institute Indemnitees") from and against any and all claims, suits, losses, damages, costs, fees, and expenses, of any kind whatsoever in law or in equity, including reasonable attorneys' fees, expert witness fees, and court costs, resulting from or arising out of or relating to Licensee's, its Affiliates', and/or other Sublicensees' and/or any other party to whom access to the Licensed Technology are provided: (a) breach of this Agreement and/or the corresponding Sublicense or other agreement relating to the Licensed Technology and/or Licensed Products; (b) exercise or practice of the rights granted hereunder, including the manufacture, sale, offer for sale, importation, keeping, or use of Licensed Products and product liability relating to the same; (c) acts or omissions of negligence or willful misconduct; and/or any order for damages, including attorneys' fees, all expenses and costs, that may be made against Research Institute Indemnitees in such proceeding.

8.2 Insurance. Licensee shall obtain and maintain at all times during the Term and after, and shall require its Sublicensees, including Affiliates, to obtain and maintain insurance sufficient to ensure all obligations to Research Institute and its Affiliates and subsidiaries hereunder, including without affecting the generality of the foregoing: (a) insurance for all statutory workers' compensation and employers' liability requirements covering any and all employees with respect to activities resulting from, arising out of or relating to this Agreement; and (b) comprehensive general liability insurance, including product liability insurance, with reputable and financially secure insurance carriers in amounts sufficient to cover their respective activities and indemnity obligations. Further without affecting the generality of the foregoing, such insurance shall: (i)

provide an appropriate and standard level of coverage considering the size of Licensee, the type of Licensed Product and standards in the industry, which in any event shall not be less than the amount required to satisfy Licensee's obligations to Research Institute Indemnitees; and (ii) include Research Institute Indemnitees as additional insureds. At Research Institute's request, Licensee shall furnish a certificate of insurance evidencing the policy's compliance herewith. Licensee is required to provide Research Institute with [**] prior written notice of cancellation or material change in such policy. Notwithstanding the foregoing, Licensee shall maintain no less than [**] dollars ($[**]) in general liability products liability coverage.

8.3 Procedure. Any Research Institute Indemnitee seeking indemnification under Section 8.1 shall provide Licensee with prompt written notice of any claim, suit, action, demand, or judgment for which indemnification is sought; provided that, a Research Institute Indemnitee's failure to do so shall not affect the rights of such Research Institute Indemnitee unless, and then only to the extent that, such delay or failure is prejudicial to or otherwise adversely affects Licensee. The Research Institute Indemnitee shall cooperate with Licensee in such defense as requested and shall permit Licensee to conduct and control such defense and the disposition of such claim, suit, or action (including all decisions relative to litigation, appeal, and settlement); provided, however, that any Research Institute Indemnitee shall have the right to retain its own counsel, at its own expense. Licensee shall keep Research Institute fully informed in writing on Licensee's actions regarding the indemnification obligations hereunder and of Licensee's defense(s) of any claim under this Article 8. Licensee shall not enter into any settlement, consent judgment, or other voluntary final disposition of any action in a manner that imposes any material obligation on, or that makes any admission of liability on behalf of Research Institute Indemnitees (including compromising the validity or enforceability of Exclusive Licensed Patents and/or Technical Information) or makes any public statements relating to Research Institute Indemnitees without Research Institute's prior written consent.

ARTICLE IX
ACKNOWLEDGEMENTS; NO USE OF NAMES OR ENDORSEMENT

9.1 Acknowledgements. Promptly after the Effective Date of this Agreement the Parties will jointly agree and issue a press release regarding this Agreement. In the absence of joint agreement, not to be unreasonably withheld, neither Party will issue a press release or other form of public announcement regarding this Agreement and activities hereunder.

9.2 No Use of Names. Except as otherwise expressly permitted by this Agreement, Licensee shall not, without the prior written consent of Research Institute, identify Research Institute, or any Affiliate of Research Institute, in any advertising or other promotional materials to be disseminated to the public or use the name of Research Institute or its Affiliates or any of their respective faculty members, employees, or students, or any trademark, service mark, trade name, or symbol owned by or associated with Research Institute, and/or any Affiliate of Research Institute, provided that, Licensee may identify Research Institute or its Affiliates as required to convey that this Agreement, and the Licensed Technology granted hereunder, exist and have been entered into, between Licensee and Research Institute. Except as otherwise expressly permitted by this Agreement, Research Institute shall not, without the prior written consent of Licensee, identify Licensee, or any Affiliate of Licensee, in any advertising or other promotional materials to be disseminated to the public or use the name of Licensee or its Affiliates or any of their respective

directors, officers or employees, or any trademark, service mark, trade name, or symbol owned by or associated with Licensee, and/or any Affiliate of Licensee, provided that, Research Institute may identify Licensee or its Affiliates as required to convey that this Agreement, and the Licensed Technology granted hereunder, exist and have been entered into, between Licensee and Research Institute.

9.3 No Endorsement. Notwithstanding anything to the contrary, Research Institute and its Affiliates do not directly or indirectly endorse any product or service provided, or to be provided, by Licensee, its Affiliates and/or Sublicensees, including the Licensed Product. Licensee its Affiliates and/or Sublicensees shall not state or imply any endorsement by Research Institute or its Affiliates or any of their employees.

ARTICLE X
CONFIDENTIALITY

10.1 Definition. The Parties agree to keep and maintain any information or materials identified as confidential by the disclosing Party ("Disclosing Party") when provided to the other Party ("Receiving Party") individually and collectively ("Confidential Information") in confidence and shall not disclose, use or otherwise make available the Confidential Information during and for five (5) years after the Term except as reasonably necessary to fulfill its obligations or exercise its rights under this Agreement and provided that any party receiving disclosure has agreed to an obligation of confidentiality and prohibition on use at least as protective as this Article 10. In no event shall Licensee or anyone receiving Confidential Information from Licensee use such Confidential Information in any manner detrimental to Research Institute, its Affiliates or their respective rights. Receiving Party has the right to disclose Confidential Information received from Disclosing Party to its Affiliates, agents and independent contractors and their respective employees under an obligation of confidentiality at least as stringent as provided for herein. Licensee remains liable for the compliance of its Sublicensees, subcontractors and any other party receiving Confidential Information from Licensee. Confidential Information disclosed under the: (a) Mutual Confidentiality Disclosure Agreement by and between the Parties dated as of September 29, 2015; (b) the Sponsored Research Agreement; or (c) any of the Ancillary Agreements shall, in each case ((a)-(c)), be deemed Confidential Information under this Agreement, and is subject to the terms and conditions of this Agreement; provided, however, Technical Information relating to NCH Technology shall be maintained by Licensee and Sublicensees, including Designated Manufacturers as a trade secret of Research Institute until such time as Research Institute authorizes otherwise in writing.

10.2 Exceptions. Confidential Information does not include any information or material that Receiving Party reasonably demonstrates:

(a) by adequate written records that it knew or possessed prior to its receipt from Disclosing Party;

(b) is in the public domain through no act or omission of the Receiving Party or anyone accessing Confidential Information therefrom;

(c) is subsequently lawfully disclosed to Receiving Party by a Third Party free of any obligations of confidentiality; or

(d) by adequate and contemporaneous written records has been independently developed by employees of the Receiving Party or its Affiliates without knowledge or use of or reliance on the Disclosing Party's Confidential Information.

Confidential Information specific to particular products or circumstances shall not be deemed to be within the exceptions stated in Section 10.2 merely if embraced by general disclosures regarding other products or circumstances. A combination of features shall not be deemed to be within the foregoing exceptions merely if the individual features of such combination qualify.

10.3 Permitted Disclosure. Notwithstanding Section 10.1 if Receiving Party is required by law, regulation or court order (including in connection with any filing with the United States Food and Drug Administration and the United States Securities and Exchange Commission) or the rules of any securities exchange to disclose the Confidential Information, it shall have the right to do so provided that Receiving Party shall (a) provide prompt written notice to Disclosing Party of the existence, terms and circumstances of such required disclosure; (b) allow the Disclosing Party to offer its objections to the production of the applicable Confidential Information; (c) cooperate with the Disclosing Party to take legally available steps to limit such disclosure, including by reasonably assisting the Disclosing Party in its efforts to obtain a protective order or other remedy of Disclosing Party's election; (d) disclose only those portions of Confidential Information that the Receiving Party is, in the opinion of its counsel, legally obligated to disclose, and (e) seek confidential treatment for all Confidential Information so disclosed.

10.4 Publication. Research Institute shall make academically reasonable efforts without an obligation to investigate to furnish Licensee with a final draft of any proposed publication, presentation or other public disclosure on a Licensed Product made by Research Institute at least [**] in advance of the submission of such proposed publication, presentation or other public disclosure in order for Licensee to review and comment thereon. Research Institute shall consider Licensee's suggestions for modifications as long as the neutrality and scientific character of the publication is not impaired. If the proposed publication, presentation or other public disclosure contains Confidential Information that is desired by Licensee to be patented, then Licensee shall have the right to require that Research Institute delay such publication, presentation or other public disclosure for a period of [**] after expiration of the [**] review period set forth above, to enable the Parties to seek protection for the information intended to be disclosed in such proposed publication, presentation or other disclosure. All proposed publications submitted to a journal or other forum of publication shall be drafted in accordance with all applicable International Committee of Medical Journal Editors ("ICMJE") guidelines. If required by the journal to which a proposed publication is submitted or requested by a Party, the publishing Party shall acknowledge the contributions of the Materials in such publication and may use the name of a Party solely for such purpose.

10.5 Publicity. Subject to Section 9.1, following the execution of this Agreement, neither Party shall issue a press release or public announcement relating to this Agreement without the prior written approval of the other Party, which approval shall not be unreasonably withheld,

conditioned or delayed, except that a Party may: (a) once a press release or other public statement is approved in writing by both Parties, make subsequent public disclosures of the information contained in such press release or other written statement without the further approval of the other Party, and (b) issue a press release or public announcement as required, in the reasonable judgment of such Party, by applicable law, including by the rules or regulations of the United States Securities and Exchange Commission or similar regulatory agency in a country other than the United States or of any stock exchange or listing entity.

ARTICLE XI
EXPIRATION & TERMINATION

11.1 Expiration. This Agreement commences on the Effective Date and, unless earlier terminated in accordance with its terms, expires at the end of the last to expire Royalty Period (the "Term"). Upon expiration (but not termination) of this Agreement and subject to compliance with the surviving rights, the licenses granted under Section 2.1.1 and Section 2.1.2 shall automatically convert to a non-exclusive, perpetual, irrevocable, fully paid-up license, limited to the Field of Use and subject to Sections 2.2-2.5.

11.2 Termination.

11.2.1 Convenience. Licensee may terminate this Agreement in its entirety or on a Licensed Product-by-Licensed Product and country-by-country basis for convenience at any time after the first anniversary of the Effective Date by giving written notice to Research Institute at least [**] prior to the effective date of termination.

11.2.2 Material Breach. A Party may terminate this Agreement immediately upon notice to the other Party if such Party is in material breach of this Agreement and such breach is not cured within [**] after written notice thereof. If either Party initiates a dispute resolution procedure under Section 12.7 to resolve a dispute or controversy regarding the material breach for which termination is being sought and is diligently pursuing such procedure, then the cure period set forth in this Section 11.2.2 will be tolled during the pendency of such dispute resolution procedure.

11.2.3 Bankruptcy. Unless prohibited by law, Research Institute may terminate this Agreement immediately without notice to Licensee in the event of: (a) the bankruptcy, insolvency (either a deficit in net worth or the inability to pay debts as they mature), or dissolution of Licensee; (b) Licensee making an assignment for the benefit of its creditors or an offer of settlement, extension, or composition to its unsecured creditors generally; or (c) the appointment of a trustee, conservator, receiver, or similar fiduciary for Licensee for substantially all of the assets of Licensee.

11.2.4 Litigation. Research Institute may immediately terminate this Agreement, unless prohibited by law, if Licensee or any Sublicensee directly or indirectly brings any litigation against Research Institute

regarding the subject matter of this Agreement unless such action or proceeding is: (a) for Research Institute's uncured material breach of a contractual obligation, recklessness or willful misconduct, (b) brought in defense of Research Institute's assertion of any Licensed Patent against Licensee or a Sublicensee or (c) brought by a Third Party that after the Effective Date acquires or is acquired by Licensee or a Sublicensee or its or their business or assets, whether by stock purchase, merger, asset purchase or otherwise, provided that such litigation commenced prior to the closing of such acquisition. In the event Research Institute is a prevailing party, Licensee agrees to promptly pay Research Institute for all costs and expenses of the suit including reasonable attorneys' fees and court costs.

11.3 Consequences of Termination.

11.3.1 Reversion of Rights. Upon the termination of this Agreement, all rights granted immediately revert to Research Institute and Licensee agrees not to practice or have practiced the Licensed Patents or the Technical Information, with the exception of any Technical Information solely in the Field of Use that Licensee can demonstrate by written evidence: (a) Licensee knew or possessed prior to its receipt from Research Institute; (b) is in the public domain through no act or omission of Licensee or anyone accessing such Technical Information therefrom; (c) is subsequently lawfully disclosed to Licensee by a Third Party free of any obligations of confidentiality; or (d) is independently developed by employees of Licensee or its Affiliates without knowledge of or access to such Technical Information. All Confidential Information of the other Party shall be returned or destruction certified, at the Disclosing Party's election provided that the Receiving Party shall be permitted to retain one copy of the Confidential Information in order to verify its compliance hereunder.

11.3.2 Sublicense Survival. Upon the termination of this Agreement, Licensee shall promptly notify its Sublicensees of such termination, and any Sublicense will be automatically revoked. However, Sublicensees shall have the right to enter into a written license agreement with Research Institute, through which such Sublicensee shall become bound to Research Institute on substantially the same terms and conditions (including financial terms) as it was bound to Licensee under the Sublicense (in view of this Agreement), but only to the extent that each such financial term is no less favorable to Research Institute than those in this Agreement; provided, however, Research Institute shall not have any obligations under the new license agreement that are greater than or inconsistent with the obligations of Research Institute under this Agreement. If any Sublicensee desires to enter into such a license agreement, it shall be wholly the responsibility of that Sublicensee to notify Research Institute of such desire within [**] after the effective date of termination of this Agreement after which Research Institute shall have no further obligations. Subject to Sublicensee's ability to comply with Section 2.4, Research Institute hereby agrees to enter into

such written license agreement, with modifications as is reasonably necessary to accommodate the functional and structural differences between Licensee and Research Institute and to carry forward obligations of Licensee under the Agreement.

11.3.3 Surviving Rights and Obligations. The termination or expiration of this Agreement does not relieve either Party of its rights and obligations that have previously accrued. Rights and obligations that by their nature prescribe continuing rights and obligations shall survive the termination or expiration of this Agreement. Without limiting the foregoing, the following provisions shall survive any termination or expiration of this Agreement: Articles 1, 7, 8, 10, 11, 12 and Article 13.

11.3.4 [**].

ARTICLE XII
MISCELLANEOUS PROVISIONS

12.1 Notices. All notices required or permitted to be given under this Agreement shall be effective when sent to the applicable Party's address set forth below or to such other address as may be designated by written notice and given in writing, with reference to this Agreement, and when: (a) delivered personally; (b) sent by electronic mail, receipt confirmed; (c) [**] after having been sent by registered or certified mail, return receipt requested, postage prepaid; or (d) [**] after deposit with a commercial overnight carrier, with written verification of receipt.

To Research Institute: Research Institute at Nationwide Children's Hospital
Attention: Director, Office of Technology Commercialization
700 Children's Drive
Columbus, Ohio 43205
Phone: [**]
Email: [**]

With Copy to: Legal Services
Nationwide Children's Hospital
700 Children's Drive
Columbus, Ohio 43205
Email: [**]

To Licensee: Sarepta Therapeutics, Inc.
215 First Street, Suite 415
Cambridge, MA 02142
Attention: General Counsel
Ty Howton
Email: [**]

With a copy (which shall not constitute notice) to:

Ropes & Gray LLP

Prudential Tower
800 Boylston Street
Boston, MA 02199
Attention: David M. McIntosh
Email: [**]

12.2 Assignment. This Agreement is personal to Licensee and may not be assigned, transferred or delegated, in whole or in part, by Licensee without the prior written consent of Research Institute, which shall not be unreasonably withheld. Provided, however, that Licensee may assign this Agreement to an Affiliate or along with all or substantially all of its assets to which this Agreement relates to any Affiliate or to any entity with which it may merge or consolidate or to which it may sell all or substantially all of its assets, without obtaining the consent of Research Institute provided that such assignee assumes in writing all of the obligations of Licensee hereunder and notice thereof if provided to Research Institute. Any attempted assignment, transfer or delegation, including any Sublicense in contravention with the terms and conditions of this Agreement shall be null and void. Research Institute has the right to assign or transfer the Licensed Patents, the Technical Information, its obligations and/or benefits hereunder and this Agreement without the consent of Licensee provided that any such assignee is bound by the terms and conditions herein. A Change of Control shall be treated like a transfer of this Agreement. This Agreement shall be binding on the Parties and their successors and assigns and shall inure to the benefit of the Parties and their permitted successors and assigns. The representations, warranties, covenants, and undertakings contained in this Agreement are for the sole benefit of the Parties and their permitted successors and assigns and shall not be construed as conferring any rights to any third party.

12.3 Entire Agreement; Amendments. This Agreement including its Exhibits contain the entire understanding of the Parties as of the Effective Date with respect to the subject matter herein and supersedes all other prior communications, agreements (including the Exclusive Option Agreement), or understandings, written or oral with respect to this Agreement subject to the surviving obligations therein and this Agreement; provided, however, the Sponsored Research Agreement will remain in effect following the Effective Date, and the Ancillary Agreements will remain in effect following the Effective Date solely to effectuate payment obligations of Licensee therein. All other terms in the Exclusive Option Agreement and Ancillary Agreements are terminated as of the Effective Date of this Agreement. The Parties may, from time to time during the Term, modify, vary or alter any of the provisions of this Agreement, but only by an instrument duly executed by authorized officials of both Parties and only if such instrument specifically states that it is an amendment to this Agreement. Each Party acknowledges that it was provided an opportunity to seek advice of counsel and as such this Agreement shall not be strictly construed for or against either Party.

12.4 Severability. The terms and conditions of this Agreement are severable, and in the event that any term or condition of this Agreement shall be determined by a court of competent jurisdiction to be invalid, illegal or unenforceable, that term or condition shall be reformed if possible, but only to the extent necessary to remove such invalidity, illegality or unenforceability in such jurisdiction and to effectuate the intent of the Parties as evidenced on the Effective Date. If reformation is not possible, then that term or condition shall be deleted and neither the validity, legality or enforceability of remaining terms and conditions nor the validity, legality or enforceability of the

deleted term or condition in any jurisdiction where it is valid, legal or enforceable shall in any way be affected or impaired thereby.

12.5 Waiver. No waiver by either Party of any term or condition of this Agreement, no matter how long continuing or how often repeated, shall be deemed a waiver of any subsequent act or omission, nor shall any delay or omission on the part of either Party to exercise any right, power, or privilege or to insist upon compliance with any term or condition of this Agreement be deemed a waiver of such right, power or privilege or excuse a similar subsequent failure to perform any such term or condition. All waivers must be in writing and signed by the Party granting such waiver.

12.6 No Agency. The relationship between the Parties is that of independent contractors. Neither Party shall be deemed to be an agent, employee, joint venturer or partner of the other and neither Party shall have any right or authority to assume or create any obligation or responsibility on behalf of the other Party or to bind that Party in any manner.

12.7 Governing Law. This Agreement shall be governed solely by the laws of the state of Ohio, without regard to any choice-of-law provisions, the Uniform Commercial Code or the International Convention on the Sale of Goods. In any litigation or arbitration arising under or relating to the terms and conditions of this Agreement, the prevailing Party or Parties shall be entitled to recover all documented costs and expenses of the suit, action or proceeding, including reasonable attorneys' fees and court and/or arbitration costs.

12.8 Jurisdiction and Forum. The state and federal courts located in Franklin County in the state of Ohio shall have exclusive jurisdiction over any claim or dispute resulting from, relating to or arising out of this Agreement. Licensee hereby irrevocably consents to the exclusive jurisdiction of such courts and irrevocably waives any claim of inconvenient forum.

12.9 Export Control. It is understood that Research Institute is subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities that may require a license from the applicable agency of the United States government and/or may require written assurances by Licensee that it shall not export data or commodities to certain foreign countries without prior approval of such agency. Research Institute neither represents that a license is required, nor that, if required, it shall be issued.

ARTICLE XIII
SPECIAL PROVISIONS

13.1 Product IND and Orphan Drug Designation. Research Institute shall initiate the transfer of title to Licensee of the Product IND and submit a written notice to FDA and any other applicable Regulatory Authorities thereof within [**] following the Effective Date. As soon as reasonably practicable following the date on which the FDA provides written acknowledgement of the transfer of the Product IND to Licensee, and subject to the terms and conditions of this Agreement, Research Institute shall transfer title and assign to Licensee the Product IND, copies (true, accurate and unredacted) of all associated correspondence with any Regulatory Authority, and copies (true, accurate and unredacted) of all associated regulatory filings or documents, in all cases in totality existing as of the date of transfer, including any Orphan Drug Designation for a Licensed Product

necessary or desirable for Licensee to undertake clinical development, including Regulatory Approvals, and commercialization of Licensed Products. Following such assignment, Licensee shall continue to have the exclusive license to the Exclusive Technical Information under Section 2.1.2 that is not assigned by the preceding sentence and Research Institute agrees to provide to Licensee as reasonably necessary all information owned by Research Institute with respect to satisfying Licensee's obligations as the sponsor of Product IND and holder of the Orphan Drug Designation. Immediately upon termination of this Agreement by Research Institute pursuant to Section 11.2.2, Licensee hereby agrees to assign and assigns all right, title an interest back to Research Institute in Product IND and/or any associated Orphan Drug Designations, copies (true, accurate and unredacted) of all associated correspondence with any Regulatory Authority, and copies (true, accurate and unredacted) of all associated regulatory filings or documents, in all cases in totality existing as of the date of such termination. For the avoidance of doubt and unless this Agreement earlier terminates, Research Institute shall not be permitted to transfer the Product IND or any associated Orphan Drug Designation, copies of any associated correspondence with any Regulatory Authority or copies of any associated regulatory filings or documents to any Third Party prior to the transfer of Product IND or the Orphan Drug Designation to Licensee in accordance with this Section 13.1. Except in connection with a permitted assignment of this Agreement in accordance with Section 12.2, Licensee shall not transfer, assign or otherwise encumber Product IND and/or any associated Orphan Drug Designations, copies of all associated correspondence with any Regulatory Authority, and copies of all associated regulatory filings or documents, in all cases in totality existing as of the date of such termination. Licensee shall execute all documents and take any lawfully requested action to effectuate any assignment back to Research Institute under this Section 13.1. Notwithstanding anything to the contrary, Research Institute, on behalf of itself, its Affiliates and entities contracting with any of the foregoing, shall have the irrevocable right to reference data filed in the Product IND and/or any other regulatory filing referring to the Technical Information but not for Licensed Product in the Field of Use.

13.2 Clinical Trial Agreement. Within [**] following the date on which Research Institute completes the transfer of title and assignment to Licensee of the Product IND pursuant to Section 13.1, the Parties shall negotiate a clinical trial agreement governing the rights and obligations of the Parties with respect to conduct of the Phase I/IIa Trial, which agreement shall have substantially the same intellectual property terms as the Sponsored Research Agreement.

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SIGNATURE PAGE FOLLOWS]

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The Parties have executed this Agreement by their duly authorized officers or representatives, in one or more counterparts, each of which shall be deemed an original but all of which taken together constitute one and the same instrument as of the Effective Date.

Research Institute AT NATIONWIDE CHILDREN'S HOSPITAL By: /s/ Matthew McFarland Matthew McFarland, Vice President, Commercial and Industry Relations Date 10/8/2018	**SAREPTA THERAPEUTICS, INC.** By: /s/ Doug Ingram Name: Doug Ingram Title: President & Chief Executive Officer Date 10/8/2018

EXHIBIT A-1
[**]

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EXHIBIT A-2

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EXHIBIT B
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EXHIBIT C
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EXHIBIT D
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EXHIBIT E

[**]

EXHIBIT F

[**]

[**] = Identified information has been excluded from this exhibit because it is both (i) information that the Company customarily and actually treats as private or confidential and (ii) is not material.

Exhibit 10.3

[**] = IDENTIFIED INFORMATION HAS BEEN EXCLUDED FROM THIS EXHIBIT BECAUSE IT IS BOTH (I) INFORMATION THAT THE COMPANY CUSTOMARILY AND ACTUALLY TREATS AS PRIVATE OR CONFIDENTIAL AND (II) IS NOT MATERIAL

FIRST AMENDMENT TO EXCLUSIVE LICENSE AGREEMENT BETWEEN THE RESEARCH INSTITUTE AT NATIONWIDE CHILDREN'S HOSPITAL AND SAREPTA THERAPEUTICS, INC.

This First Amendment ("First Amendment") is entered into as of May 29, 2019, to amend the Exclusive License Agreement (the "License Agreement") entered into as of October 8, 2018, by and between The Research Institute at Nationwide Children's Hospital ("the Research Institute") and Sarepta Therapeutics, Inc. ("Licensee").

WHEREAS, under the License Agreement, Sarepta has a license to the Exclusive Technical Information (as that term is defined in the License Agreement) (as well as other intellectual property, information and materials).

WHEREAS, to further the aims of the License Agreement and facilitate Sarepta's ability to develop and market the Licensed Technology at the earliest possible time to ensure availability for public use and benefit, the Parties desire Research Institute to transfer additional materials, which shall be included in the "Exclusive Technical Information" as defined in the License Agreement. Such additional information is listed in Schedule 1 hereto. To the extent that the Research Institute has not already provided the materials listed in Schedule 1 to Sarepta, it will do so in accordance with the terms and conditions of this First Amendment and the License Agreement.

NOW THEREFORE, for good and valuable consideration, the receipt of which is hereby acknowledged by the parties, the parties hereto agree that the Agreement is amended by this First Amendment as follows:

1. Exhibit E of the License Agreement is amended to include the additional Exclusive Technical Information listed in Schedule 1 hereto. Sarepta agrees to use the Exclusive Technical Information listed in Schedule 1 in compliance with all applicable laws and regulations. To the extent such materials constitute materials derived from human research subjects, Sarepta will use such materials solely as allowed by the Informed Consent document signed by the research subjects.

2. Promptly after the effective date of this First Amendment, Research Institute agrees to send to Sarepta or its designated Affiliate the Exclusive Technical Information listed in Schedule 1 hereto, to the extent such material has not already been transferred under the License Agreement or another agreement between the parties.

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3. Except for the addition of the items listed in Schedule 1 as Exclusive Technical Information and terms covering the sharing and use of the items listed in Schedule 1, all other terms and conditions of the License Agreement shall remain unchanged and in full force and effect.

4. This First Amendment may be executed in counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. A signed copy of this First Amendment delivered by email or other means of electronic transmission shall be deemed to have the same effect as delivery of an original signed copy of this First Amendment.

IN WITNESS WHEREOF, the parties hereto have caused this First Amendment to be executed by their duly authorized representatives as of the date first set forth above.

SAREPTA THERAPEUTICS, INC.		**THE RESEARCH INSTITUTE AT NATIONAL CHILDREN'S HOSPITAL**	
By:	/s/ Ty Howton	By:	/s/ Matthew McFarland
Name:	Ty Howton	Name:	Matthew McFarland Ph.D. RPh
Title:	EVP, General Counsel	Title:	VP Commercialization and Industry Relations.
Date:	June 3, 2019	Date:	5/30/2019

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SCHEDULE 1

[**]

[**] = Identified information has been excluded from this exhibit because it is both (i) information that the Company customarily and actually treats as private or confidential and (ii) is not material.

Exhibit 10.4

SECOND AMENDMENT TO EXCLUSIVE LICENSE AGREEMENT
BETWEEN
THE RESEARCH INSTITUTE AT NATIONWIDE CHILDREN'S HOSPITAL
AND
SAREPTA THERAPEUTICS THREE LLC
(as successor to SAREPTA THERAPEUTICS, INC.)

This Second Amendment ("Second Amendment") is entered into as of July 11, 2023 ("Second Amendment Date"), by and between the Research Institute at Nationwide Children's Hospital ("Research Institute") and Sarepta Therapeutics Three LLC ("Licensee").

WHEREAS, Research Institute and Sarepta Therapeutics, Inc. entered into to the Exclusive License Agreement effective as of October 8, 2018, and amended on May 29, 2019 by a First Amendment (as amended, the "Agreement").

WHEREAS, Sarepta Therapeutics, Inc. assigned the Agreement to Licensee, its Affiliate.

WHEREAS, the Parties desire to amend the Agreement as set forth herein.

NOW THEREFORE, for good and valuable consideration, the receipt of which is hereby acknowledged, the Parties hereto agree that the Agreement is amended by this Second Amendment as follows:

1. Section 1.1.6 of the Agreement is deleted in its entirety and replaced with the following:

   1.6.6. "Designated Manufacturer" shall mean Brammer Bio MA, LLC and/or Catalent Maryland, Inc., together with their affiliates, or another entity approved in writing by Research Institute.

2. Section 2.1.5 of the Agreement (Additional Designated Manufacturer) is hereby deleted in its entirety.

3. All capitalized terms used but not defined herein shall have the meaning set forth in the Agreement.

4. Except as expressly set forth herein, all other terms and conditions of the Agreement shall remain unchanged and in full force and effect.

5. This Second Amendment may be executed in counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. A signed copy of this Second Amendment delivered by email or other means of electronic transmission shall be deemed to have the same effect as delivery of an original signed copy of this Second Amendment.

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IN WITNESS WHEREOF, the Parties hereto have caused this Second Amendment to be executed by their duly authorized representatives as of the Second Amendment Date.

SAREPTA THERAPEUTICS THREE LLC	**THE RESEARCH INSTITUTE AT NATIONWIDE CHILDREN'S HOSPITAL**
By: /s/ Adam Hopkin	By: /s/ Matthew McFarland
Name: Adam Hopkin	Name: Matthew McFarland PhD, RPh
Title: Manager	Title: VP Commercialization & Industry Relations
Date: 13 July 2023	Date: July 13, 2023

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FIRST AMENDMENT TO THE

AMENDED AND RESTATED

LEAD DMD PRODUCT MANUFACTURING & SUPPLY AGREEMENT

This first amendment to the Amended and Restated Lead DMD Product Manufacturing & Supply Agreement (**"First Amendment"**) by and between Sarepta Therapeutics Three, LLC ("**Sarepta**"), and Catalent Maryland, Inc. ("**Catalent**").

WHEREAS, Sarepta and Catalent entered into that certain Amended and Restated Lead DMD Product Manufacturing & Supply Agreement dated November 28, 2022 (the "**Agreement**"); and

WHEREAS, Sarepta and Catalent desire to amend the Agreement to correct a scrivener's error therein;

NOW, THEREFORE, the Parties agree as follows:

1. Correction to the Preamble. The preamble to the Agreement identifies Sarepta Therapeutics, Inc. as "Sarepta," a Party (as that term is defined in the Agreement) to the Agreement. This identification was a scrivener's error, and the Party identified therein as "Sarepta" is Sarepta Therapeutics Three, LLC, as listed on the cover sheet and signature page of the Agreement. Sarepta Therapeutics, Inc. is not and never was intended to be a Party to the Agreement. Accordingly, the first sentence of the preamble of the Agreement is hereby replaced in its entirety with this sentence: "**THIS AMENDED AND RESTATED LEAD DMD PRODUCT MANUFACTURING & SUPPLY AGREEMENT** (this "**Agreement**"), dated as of the 28th day of November, 2022 (the "**Effective Date**"), is entered into by and among **SAREPTA THERAPEUTICS THREE, LLC**, a corporation organized and existing under the Laws of Delaware and having a place of business at 215 First Street, Boston, Massachusetts 02142, ("**Sarepta**") and **CATALENT MARYLAND, INC.** (formerly **PARAGON BIOSERVICES, INC.**), a corporation organized and existing under the Laws of Delaware and having a place of business at 801 West Baltimore Street, Suite 302, Baltimore, Maryland 21201 ("**Catalent**").

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2. No Other Variations. Except as specifically amended herein, all other terms and conditions of the Agreement remain in full force and effect and shall apply to the construction of this First Amendment.

3. Counterparts. This First Amendment may be executed in one or more counterparts, each of which will be deemed an original but all of which together will constitute one and the same instrument. Any photocopy, facsimile or electronic reproduction of the executed First Amendment shall constitute an original.

IN WITNESS WHEREOF, the parties have caused their respective duly authorized Representatives to execute this First Amendment effective as of November 28, 2022.

CATALENT MARYLAND, INC	**SAREPTA THERAPEUTICS THREE, LLC**
By: /s/ Manja Boerman	By: /s/ Adam Hopkin
Name: Manja Boerman	Name: Adam Hopkin
Title: President, Cell & Gene Therapy	Title: Manager

EXHIBIT 31.1

CERTIFICATION

I, Douglas S. Ingram, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q 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.

August 2, 2023

/s/ DOUGLAS S. INGRAM

Douglas S. Ingram
President and Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

CERTIFICATION

I, Ian M. Estepan, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q 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.

August 2, 2023

/s/ IAN M. ESTEPAN

Ian M. Estepan
Executive Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

EXHIBIT 32.1

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Douglas S. Ingram, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended June 30, 2023, 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 Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

August 2, 2023

/s/ DOUGLAS S. INGRAM

Douglas S. Ingram
President and Chief Executive Officer
(Principal Executive Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.

EXHIBIT 32.2

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Ian M. Estepan, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended June 30, 2023, 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 Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

August 2, 2023

/s/ IAN M. ESTEPAN

Ian M. Estepan
Executive Vice President, Chief Financial Officer
(Principal Financial and Accounting Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q 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.