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, 2018

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

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a 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 (Check one):

Large accelerated filer	☒	Accelerated filer	☐
Non-accelerated filer	☐ (Do not check if a smaller reporting company)	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	**66,442,402**
(Class)	**(Outstanding as of August 3, 2018)**

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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, 2018	As of December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 410,375	$ 599,691
Short-term investments	538,769	479,369
Accounts receivable	42,985	29,468
Inventory	104,126	83,605
Other current assets	42,989	36,511
Total current assets	1,139,244	1,228,644
Property and equipment, net of accumulated depreciation of $22,124 and $18,022 as of June 30, 2018 and December 31, 2017, respectively	57,624	43,156
Intangible assets, net of accumulated amortization of $5,100 and $4,145 as of June 30, 2018 and December 31, 2017, respectively	14,857	14,355
Other assets	35,435	21,809
Total assets	$ 1,247,160	$ 1,307,964
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 17,382	$ 8,467
Accrued expenses	72,477	68,982
Current portion of long-term debt	9,514	6,175
Deferred revenue	3,303	3,316
Other current liabilities	2,011	1,392
Total current liabilities	104,687	88,332
Long-term debt	429,925	424,876
Deferred rent and other	13,501	5,539
Total liabilities	548,113	518,747
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, 99,000,000 shares authorized; 66,346,248 and 64,791,670 issued and outstanding at June 30, 2018 and December 31, 2017, respectively	7	6
Additional paid-in capital	2,061,039	2,006,598
Accumulated other comprehensive loss	(361)	(379)
Accumulated deficit	(1,361,638)	(1,217,008)
Total stockholders' equity	699,047	789,217
Total liabilities and stockholders' equity	$ 1,247,160	$ 1,307,964

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,	
	2018	2017	2018	2017
Revenues:				
Product, net	$ 73,529	$ 35,011	$ 138,133	$ 51,353
Total revenues	73,529	35,011	138,133	51,353
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	$ 6,735	506	$ 12,317	729
Research and development	122,848	58,908	169,052	88,027
Selling, general and administrative	47,156	36,069	90,497	62,285
EXONDYS 51 litigation and license charges	—	2,839	—	2,839
Amortization of in-licensed rights	217	28	433	57
Total costs and expenses	176,956	98,350	272,299	153,937
Operating loss	(103,427)	(63,339)	(134,166)	(102,584)
Other (loss) income:				
Gain from sale of Priority Review Voucher	—	—	—	125,000
Interest (expense) income and other, net	(5,218)	184	(9,703)	519
Other (loss) income	(5,218)	184	(9,703)	125,519
(Loss) income before income tax expense (benefit)	(108,645)	(63,155)	(143,869)	22,935
Income tax expense (benefit)	622	(109)	761	1,891
Net (loss) income	(109,267)	(63,046)	(144,630)	21,044
Other comprehensive (loss) income:				
Unrealized gain on cash equivalents and short-term investments	282	17	18	82
Total other comprehensive income	282	17	18	82
Comprehensive (loss) income	$ (108,985)	$ (63,029)	$ (144,612)	$ 21,126
Net (loss) income per share				
Basic (loss) earnings per share	$ (1.67)	$ (1.15)	$ (2.22)	$ 0.38
Diluted (loss) earnings per share	$ (1.67)	$ (1.15)	$ (2.22)	$ 0.37
Weighted average number of shares of common stock used in computing:				
Basic (loss) earnings per share	65,484	54,976	65,060	54,913
Diluted (loss) earnings per share	65,484	54,976	65,060	56,176

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,	
	2018	**2017**
Cash flows from operating activities:		
Net (loss) income	$ (144,630)	$ 21,044
Adjustments to reconcile net (loss) income to cash flows from operating activities:		
Gain from sale of Priority Review Voucher	—	(125,000)
Depreciation and amortization	5,125	3,409
Amortization of investment discount	(2,828)	(260)
Non-cash interest expense	9,958	117
Loss on disposal of assets	37	604
Stock-based compensation	25,805	16,177
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable	(13,517)	(12,563)
Net increase in inventory	(20,521)	(28,941)
Net increase in other assets	(29,866)	(9,285)
Net increase (decrease) in accounts payable, accrued expenses, deferred revenue and other liabilities	17,515	(8,337)
Net cash used in operating activities	(152,922)	(143,035)
Cash flows from investing activities:		
Purchase of property and equipment	(20,863)	(7,336)
Purchase of intangible assets	(1,556)	(1,601)
Purchase of available-for-sale securities	(295,823)	(100,348)
Proceeds from sale of Priority Review Voucher	—	125,000
Purchases of restricted investment	(353)	—
Maturity of restricted investment	—	10,695
Maturity of available-for-sale securities	249,243	163,521
Net cash (used in) provided by investing activities	(69,352)	189,931
Cash flows from financing activities:		
Proceeds from revolving line of credit	173,354	—
Payments on mortgage loans	(1,265)	—
Payments on revolving line of credit	(173,653)	—
Proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program	34,386	4,086
Repayments of long-term debt	—	(5,054)
Net cash provided by (used in) financing activities	32,822	(968)
(Decrease) increase in cash and cash equivalents	(189,452)	45,928
Cash, cash equivalents and restricted cash:		
Beginning of period	599,827	122,556
End of period	$ 410,375	$ 168,484
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 410,375	$ 168,348
Restricted cash in other assets	—	136
Total cash, cash equivalents and restricted cash	$ 410,375	$ 168,484
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 6,058	$ 538
Supplemental schedule of non-cash investing activities and financing activities:		
Shares withheld for taxes	$ 5,750	$ 309
Reclassification of long term investments to short term investments	$ 9,980	$ —
Intangible assets included in accrued expenses	$ 294	$ 265
Asset held for sale	$ —	$ 1,529
Accrual for debt issuance costs related to the term loans	$ 600	$ 400
Property and equipment included in accrued expenses	$ 289	$ —

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 the discovery and development of unique RNA-targeted therapeutics, gene therapy and other genetic medicine approaches for the treatment of rare neuromuscular diseases. Applying its proprietary, highly-differentiated and innovative platform technologies, the Company is able to target a broad range of diseases and disorders. Its first commercial product in the U.S., EXONDYS 51® (eteplirsen) Injection ("EXONDYS 51"), was granted accelerated approval by the United States Food and Drug Administration ("FDA") on September 19, 2016. EXONDYS 51 is indicated for the treatment of Duchenne muscular dystrophy ("DMD") in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

In addition to advancing its exon-skipping product candidates for DMD, including eteplirsen, golodirsen, casimersen and SRP-5051, the Company is working with several strategic partners under various agreements to research and develop multiple treatment approaches to DMD, which include Nationwide Children's Hospital, Genethon, Duke University and Summit (Oxford) Ltd. ("Summit").

In November 2016, the Company submitted a marketing authorization application ("MAA") for eteplirsen to the European Medicines Agency ("EMA") and the application was validated in December 2016. On June 1, 2018, the Committee for Medicinal Products for Human Use ("CHMP") of the EMA, adopted a negative opinion for eteplirsen. The Company has requested a re-examination of the opinion, and that a Scientific Advisory Group ("SAG") on DMD be called so that neuromuscular specialists, experienced with working with treatments for these patients, can provide expert guidance and insight into, among other things, the validity of the external controls used and the importance of certain functional endpoints, including for instance, the relevance of meaningful slowing pulmonary decline in patients with this disease that is difficult to treat. The re-examination process is expected to be completed by year-end 2018.

The Company has also initiated a market access program ("MAP") for eteplirsen in select countries in Europe, North America, South America and Asia where it currently has not been approved. The MAP provides a mechanism through which physicians can prescribe eteplirsen, within their professional responsibility, to patients who meet pre-specified medical and other criteria and can secure funding. The Company commenced shipments through the MAP. In addition, the Company contracted with third party distributors and service providers to distribute eteplirsen in certain areas outside the U.S., such as Israel and certain countries in the Middle East, on a named patient basis.

As of June 30, 2018, the Company had approximately $950.2 million of cash, cash equivalents and investments, consisting of $410.4 million of cash and cash equivalents, $538.8 million of short-term investments, and $1.0 million of restricted investment. 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 additional government contracts and establish collaborations with or license its technology to other companies.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES AND RECENT ACCOUNTING PRONOUNCEMENTS

Basis of Presentation

The accompanying 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 Therapeutics, Inc. and its wholly-owned subsidiaries. All intercompany transactions between and among its consolidated subsidiaries have been eliminated. Management has determined that the Company operates in one segment: discovering, developing, manufacturing and delivering therapies to patients with DMD. The Company's CEO, as the chief operating decision-maker, manages and allocates resources to the operations of the Company on a total company basis. The Company's research and development organization is responsible for the research and discovery of new product candidates and supports development and registration efforts for potential future products. The Company's supply chain organization manages the development of the manufacturing processes, clinical trial supply and commercial product supply. The Company's commercial organization is responsible for commercialization of EXONDYS 51 in the U.S. and internationally. The Company is supported by other back-office general and administration functions. Consistent with this decision-making process, the Company's CEO uses consolidated, single-segment financial information for purposes of evaluating performance, forecasting future period financial results, allocating resources and setting incentive targets.

Estimates and Uncertainties

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

Concentration of Credit Risk

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

As of June 30, 2018, the majority of the Company's accounts receivable arose from product sales in the U.S. and all customers have standard payment terms which generally require payment within 30 to 60 days. Outside of the U.S., the payment terms range between 45 and 120 days. Three individual customers accounted for 44%, 35% and 19% of net product revenues for the six months ended June 30, 2018 and 61%, 26% and 11% of accounts receivable from product sales as of June 30, 2018. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profile. As of June 30, 2018, the Company believes that such customers are of high credit quality.

As of June 30, 2018 the Company's cash equivalents and investments were concentrated at a single financial institution, 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 institution.

Significant Accounting Policies

For details about the Company's accounting policies, please read *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* of the Annual Report on Form 10-K for the year ended December 31, 2017.

The Company has adopted Accounting Standards Codification Topic 606, "Revenue from Contracts with Customers" ("ASC 606") effective as of January 1, 2018. The Company has chosen to use the full retrospective transition method, under which it is required to revise its consolidated financial statements for the years ended December 31, 2016 and 2017 as well as any applicable interim periods within those years, as if ASC 606 had been effective for those periods. Under ASC 606, the Company recognizes revenue when a customer obtains control of promised goods or services, in an amount that reflects the consideration which the Company expects to receive in exchange for the goods or services provided. To determine revenue recognition for arrangements within the scope of ASC 606, the Company performs the following five steps: (1) identify the contracts with a customer; (2) identify the performance obligations in the contract; (3) determine the transaction price; (4) allocate the transaction price to the performance obligations in the contract; and (5) recognize revenue when or as the entity satisfies a performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, the Company assesses the goods or services promised within each contract and determines those that are performance obligations, and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when or as the performance obligation is satisfied. For all contracts that fall into the scope of ASC 606, only one performance obligation has been identified by the Company: to timely deliver drug products to the customer's designated warehouses.

Product Revenues

The Company distributes its product principally through a limited number of specialty distributor and specialty pharmacies in the U.S. and certain distributors in the European Union ("EU"), Israel and Middle East (collectively, "Customers"). The Customers subsequently resell the product to patients and health care providers. The Company provides no right of return to the Customers except in cases of shipping error or product defect. Product revenues are recognized when the Customers take control of the product, which typically occurs upon delivery to the Customers. For both the three and six months ended June 30, 2018, the majority of the revenues recognized were generated by the specialty distributor and specialty pharmacies in the U.S.

Variable Consideration

Product revenues are recorded at the net sales price ("transaction price") which includes estimated reserves for variable consideration, such as Medicaid rebates, governmental chargebacks, including Public Health Service ("PHS") chargebacks, prompt payment 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 payment is required by the Company) or a current liability (if a payment is required by the Company). These reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the contracts. Additional details relating to variable consideration follows:

- Medicaid rebates relate to the Company's estimated obligations to states under established reimbursement arrangements. Rebate reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a liability which is included in accrued expenses.
- Governmental chargebacks, including PHS chargebacks, relate to the Company's estimated obligations resulting from contractual commitments to sell products to qualified healthcare providers at prices lower than the list prices that the Company charges to wholesalers. The wholesaler charges the Company for the difference between what the wholesaler pays for the products and the ultimate selling price to the qualified healthcare providers. Chargeback reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable. Chargeback amounts are generally determined at the time of resale to the qualified healthcare provider from the wholesaler, and the Company generally issues credits for such amounts within a few weeks of receiving notification of resale from the wholesaler.
- Prompt payment discounts relate to the Company's estimated obligations for credits to be granted to a specialty pharmacy for remitting payment on its purchases within established incentive periods. Reserves for prompt payment discounts are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and accounts receivable.
- Co-pay assistance relates to financial assistance provided to qualified patients, whereby the Company may assist them with prescription drug co-payments required by the patient's insurance provider. Reserves for co-pay assistance are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a liability which is included in accrued expenses.
- Distribution fees relate to fees paid to Customers in the distribution channel that provide the Company with inventory management, data and distribution services and are generally accounted for as a reduction of revenue. To the extent that the services received are distinct from the Company's sale of products to the Customer, these payments are accounted for as selling, general and administrative expenses.

The impact of adopting ASC 606 was not material. There have not been any other material changes to the Company's accounting policies as of June 30, 2018.

Recent Accounting Pronouncements

In February 2016, the Financial Accounting Standards Board ("FASB") issued ASU No. 2016-02, "*Leases (Topic 842)*", which supersedes Topic 840, "*Leases*". Under the new guidance, a lessee should recognize assets and liabilities that arise from its leases and disclose qualitative and quantitative information about its leasing arrangements. In transition, lessees and lessors are required to recognize and measure leases at the beginning of the earliest period presented using a modified retrospective approach. ASU No. 2016-02 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The adoption of this standard is expected to have an impact on the amount of the Company's assets and liabilities. As of June 30, 2018, the Company has not elected to early adopt this guidance or determined the effect that the adoption of this guidance will have on its consolidated financial statements.

In March 2017, the FASB issued ASU No. 2017-08, "*Receivables - Nonrefundable Fees and Other Costs (Subtopic 310-20): Premium Amortization on Purchased Callable Debt Securities*". This new standard amends the amortization period for certain purchased callable debt securities held at a premium by shortening the amortization period for the premium to the earliest call date. ASU No. 2017-02 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted. As of June 30, 2018, the Company is currently evaluating the potential impact that this new standard may have on its financial position and results of operations.

In June 2018, the FASB issued ASU 2018-07, "*Compensation - Stock Compensation (Topic 718), Improvements to Nonemployee Share-Based Payment Accounting*." This ASU expands the scope of Topic 718 to include share based payment transactions for acquiring goods and services from nonemployees. An entity should apply the requirements of Topic 718 to nonemployee awards except for specific guidance on inputs to an option pricing model and the attribution of cost. ASU No. 2018-07

will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted, although no earlier than the adoption date of Topic 606. The Company elected to early adopt this ASU in the quarter ended June 30, 2018, which did not have a material impact on its consolidated financial statements.

3. COLLABORATION, LICENSE AND MANUFACTURING AGREEMENTS

Myonexus Warrant Agreement

In May 2018, the Company entered into a Warrant to Purchase Common Stock Agreement ("Warrant Agreement") with Myonexus Therapeutics, Inc. ("Myonexus"). Pursuant to the terms of the Warrant Agreement, the Company made an up-front payment of $60.0 million to purchase an exclusive option to acquire Myonexus at a pre-negotiated, fixed price plus sales-related and regulatory-related contingent payments. Prior to the exercise of the option to acquire Myonexus, the Company may be required to make additional development milestone payments to Myonexus of up to $45.0 million over an approximately two-year evaluation period.

The up-front payment of $60.0 million to Myonexus provides the Company with rights to potential future benefits associated with Myonexus's ongoing research and development activities, which have not reached technological feasibility and have no alternative future use. Accordingly, the up-front payment of $60.0 million was expensed as incurred and classified as research and development expense in the accompanying unaudited condensed consolidated statements of operations and comprehensive loss during the three and six months ended June 30, 2018. The additional development milestones payments of up to $45.0 million will also be recorded to research and development expense when (and if) incurred.

The Company considered whether it would have to consolidate the operations of Myonexus and concluded that, while Myonexus is a variable interest entity, the Company is not the primary beneficiary as it does not have the power to direct the activities that would most significantly impact the economic performance of Myonexus.

Brammer Manufacturing Agreement

In June 2018, the Company entered into a Development, Commercial Manufacturing and Supply Agreement ("Brammer Manufacturing Agreement"), with Brammer Bio MA, LLC ("Brammer"). Pursuant to the terms of the Brammer Manufacturing Agreement, Brammer agreed to provide the Company with access to clinical and commercial manufacturing capacity for its gene therapy programs.

As part of the Brammer Manufacturing Agreement, the Company will purchase product in batches from Brammer, subject to minimum and maximum annual purchase requirements. Further, the Company: (i) was required to make a $20.0 million advance payment to Brammer upon execution of the agreement, (ii) is required to make two non-refundable payments of $5.0 million each to Brammer in the third and fourth quarter of 2018 to be used in the specification, selection, and procurement of the related process equipment to be utilized under the agreement, and (iii) is required to make a $10.0 million quarterly capacity access fee payment to Brammer throughout the term of the agreement. However, through June 30, 2019, a reduced quarterly capacity access fee will be in effect as Brammer works towards achieving full capacity at its facility. In addition, one-tenth of the $20.0 million advance payment will be applied as a credit to the quarterly capacity access fees due and payable from July 1, 2019 through December 31, 2021, resulting in a net capacity access fee of $8.0 million.

The term of the Brammer Manufacturing Agreement will continue for a period of six years following the first regulatory approval of a product manufactured under the agreement. The term will automatically renew for successive two years unless the Company notifies Brammer of its intention not to renew (no less than twenty-four months prior to the expiration of the term). The Company also has the ability to terminate the agreement prior to expiration but would be required to continue remitting capacity access fees to Brammer for up to eight additional quarters.

The Company has determined that the Brammer Manufacturing Agreement does not contain an embedded lease because it does not convey the right to control the use of the facility or related equipment. This conclusion was based on the Company's inability or right to control physical access to Brammer's facility and the related equipment, and the ability of one or more parties, other than the Company, to take more than a minor amount of the output that will be produced during the term of the agreement.

As of June 30, 2018, the $20.0 million advance payment was recorded as an other non-current asset in the accompanying unaudited condensed consolidated balance sheet. The advance payment will be amortized over its expected economic benefit to research and development expense, prior to regulatory approval of the related product, commencing upon the first batch delivery to the Company. Upon regulatory approval, amortization expense will be classified to cost of sales. In the event the Company does not expect services under the Brammer Manufacturing Agreement to be rendered, the capitalized advance payment will be charged to expense.

The two $5.0 million process equipment fee payments will also be capitalized as an other asset when paid and amortized to expense in a manner similar to the advance payment described above. Capacity access fee payments made prior to the first batch delivery to the Company will also be capitalized as an other asset when paid and amortized to expense in a manner similar to the advance payment described above. Capacity access fee payments made subsequent to the first batch delivery to the Company will be expensed as incurred to research and development expense, prior to regulatory approval of the related product. Upon regulatory approval, the expense associated with capacity access fee payments will be classified to cost of sales.

4. FAIR VALUE MEASUREMENTS

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

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

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

	Fair Value Measurement as of June 30, 2018			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 211,197	$ 211,197	$ —	$ —
Commercial paper	189,622	—	189,622	—
Government and government agency bonds	278,512	278,512	—	—
Corporate bonds	85,594	85,594	—	—
Certificates of deposit	$ 1,001	1,001	—	—
Total	$ 765,926	$ 576,304	$ 189,622	$ —

	Fair Value Measurement as of December 31, 2017			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 352,370	$ 352,370	$ —	$ —
Commercial paper	133,368	—	133,368	—
Government and government agency bonds	294,717	284,745	9,972	—
Corporate bonds	127,956	127,956	—	—
Certificates of deposit	648	648	—	—
Total	$ 909,059	$ 765,719	$ 143,340	$ —

The Company's assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds, government and government agency bonds, corporate bonds and certificates of deposit. Certain of the government and government agency bonds and corporate bonds are publically traded fixed income securities and are presented as cash equivalents on the unaudited condensed consolidated balance sheets as of June 30, 2018.

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

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

5. 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, 2018	As of December 31, 2017
	(in thousands)	
Money market funds	$ 211,197	$ 352,370
Corporate bonds	14,959	16,720
Government and government agency bonds	—	49,972
Total	$ 226,156	$ 419,062

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 June 30, 2018 and December 31, 2017 was approximately two and seven months, respectively.

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

	As of June 30, 2018			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 395,416	$ —	$ —	395,416
Commercial paper	189,622	—	—	189,622
Government and government agency bonds	278,680	5	(173)	278,512
Corporate bonds	85,787	—	(193)	85,594
Total	$ 949,505	$ 5	$ (366)	949,144
As reported:				
Cash and cash equivalents	$ 410,375	$ —	$ —	410,375
Short-term investments	539,130	5	(366)	538,769
Total	$ 949,505	$ 5	$ (366)	949,144

	As of December 31, 2017			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 532,999	$ —	$ —	$ 532,999
Commercial paper - current	133,368	—	—	133,368
Government and government agency bonds - current	294,915	2	(200)	294,717
Corporate bonds				
Current	118,121	—	(145)	117,976
Non-current	10,016	—	(36)	9,980
Total	$ 1,089,419	$ 2	$ (381)	$ 1,089,040
As reported:				
Cash and cash equivalents	$ 599,698	$ 2	$ (9)	$ 599,691
Short-term investments	479,705	—	(336)	479,369
Long-term investments	10,016	—	(36)	9,980
Total	$ 1,089,419	$ 2	$ (381)	$ 1,089,040

6. ACCOUNTS RECEIVABLE AND RESERVES FOR PRODUCT SALES

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

The accounts receivable from product sales represents receivables due from the Company's specialty distributor and specialty pharmacies in the U.S. as well as certain distributors in the EU, Israel and the Middle East. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profiles. The Company provides reserves against trade receivables for estimated losses that may result from a customer's inability to pay. Amounts determined to be uncollectible are written-off against the established reserve. As of June 30, 2018, the credit profiles for the Company's customers are deemed to be in good standing and write-offs of accounts receivable are not considered necessary. Historically, no accounts receivable amounts related to government research contracts and other grants have been written off and, thus, an allowance for doubtful accounts receivable related to government research contracts and other grants is not considered necessary.

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

	As of June 30, 2018	As of December 31, 2017
	(in thousands)	
Product sales, net of discounts and allowances	$ 42,056	$ 28,539
Government contract receivables	929	929
Total accounts receivable	$ 42,985	$ 29,468

The balance for government contract receivables for both periods presented is subject to government audit and will not be collected until the completion of the audit.

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

	Chargebacks	Rebates	Prompt Pay	Other	Total
	(in thousands)				
Balance, as of December 31, 2017	$ 995	$ 6,959	$ 169	$ 464	$ 8,587
Provision	6,490	11,178	1,094	2,460	21,222
Payments/credits	(6,622)	(1,978)	(917)	(1,813)	(11,330)
Balance, as of June 30, 2018	$ 863	$ 16,159	$ 346	$ 1,111	$ 18,479

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

	As of June 30, 2018	As of December 31, 2017
	(in thousands)	
Reduction to accounts receivable	$ 1,612	$ 1,285
Component of accrued expenses	16,867	7,302
Total reserves	$ 18,479	$ 8,587

7. INVENTORY

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis. The Company capitalizes inventory costs associated with products following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. EXONDYS 51 which may be used in clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes. The following table summarizes the components of the Company's inventory for the period indicated:

	As of June 30, 2018	As of December 31, 2017
	(in thousands)	
Raw materials	$ 64,525	$ 53,875
Work in progress	37,107	27,442
Finished goods	2,494	2,288
Total inventory	$ 104,126	$ 83,605

The Company periodically reviews its inventories for excess amounts or obsolescence and writes down obsolete or otherwise unmarketable inventory to its estimated net realizable value. Additionally, though the Company's product is subject to strict quality control and monitoring which it performs throughout the manufacturing processes, certain batches or units of product may not meet quality specifications resulting in a charge to cost of sales.

8. OTHER CURRENT ASSETS AND OTHER NON-CURRENT ASSETS

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

	As of June 30, 2018	As of December 31, 2017
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 15,338	$ 18,650
Leasehold improvement receivable	10,409	—
Prepaid clinical and preclinical expenses	7,286	5,175
Prepaid maintenance and license fees	2,745	1,711
Prepaid research expenses	2,472	2,896
Prepaid commercial expenses	374	1,589
Asset held for sale	—	1,501
Other prepaids	2,497	2,726
Other	1,868	2,263
Total other current assets	$ 42,989	$ 36,511

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

	As of June 30, 2018	As of December 31, 2017
	(in thousands)	
Manufacturing-related deposits	$ 22,633	$ —
Prepaid clinical expenses	8,680	7,488
Alternative minimum tax credit	2,881	3,315
Restricted investment	1,001	784
Long-term available-for-sale securities	—	9,980
Other	240	242
Total other non-current assets	$ 35,435	$ 21,809

9. ACCRUED EXPENSES

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

	As of June 30, 2018	As of December 31, 2017
	(in thousands)	
Accrued employee compensation costs	$ 17,949	$ 14,402
Product revenue related reserves	16,867	7,302
Accrued clinical and preclinical costs	11,068	15,975
Accrued contract manufacturing costs	8,421	14,019
Accrued professional fees	7,578	6,794
Accrued BioMarin royalties	3,695	2,846
Accrued collaboration cost sharing	2,699	—
Accrued interest expense	1,251	1,291
Accrued research costs	729	401
Accrued income taxes	523	943
Accrued property and equipment	289	2,525
Other	1,408	2,484
Total accrued expenses	$ 72,477	$ 68,982

10. INDEBTEDNESS

2024 Convertible Notes

In November 2017, the Company issued $570.0 million senior notes due on November 15, 2024 (the "2024 Notes"). The 2024 Notes were issued at face value and bear interest at the rate of 1.50% per annum, payable semi-annually in cash on each May 15 and November 15, commencing on May 15, 2018. Upon conversion, the Company may pay cash, shares of its common stock or a combination of cash and stock, as determined by the Company in its discretion. The 2024 Notes may be convertible into 7,763,552 shares of the Company's common stock under certain circumstances prior to maturity at a conversion rate of 13.621 shares per $1,000 principal amount of the 2024 Notes, which represents a conversion price of $73.42 per share. The Company recorded a total debt discount of $171.8 million upon issuance of the 2024 Notes, consisting of an equity component of $161.2 million and debt issuance costs of $10.6 million. The debt discount is being amortized under the effective interest method and recorded as additional non-cash interest expense over the life of the 2024 Notes. The effective interest rate on the liability component of the 2024 Notes for the year ended December 31, 2017 was 6.9%. The fair value of the 2024 Notes is $1.1 billion as of June 30, 2018. It is based on open market trades and is classified as level 1 in the fair value hierarchy.

Term Loan

In July 2017, the Company entered into an amended and restated credit agreement (the "Amended and Restated Credit and Security Agreement") which provides a term loan ("July 2017 Term Loan") of $60.0 million with MidCap Financial Trust ("MidCap"). Borrowings under the Amended and Restated Credit and Security Agreement bear interest at a rate per annum equal to 6.25%, plus the one-month London Interbank Offered Rate ("LIBOR"). Commencing on July 1, 2018, and continuing for the remaining thirty six months of the facility, the Company will be required to make monthly principal payments of approximately $0.8 million, set forth in the Amended and Restated Credit and Security Agreement, subject to certain adjustments as described therein. The facility matures in July 2021. The Company was in compliance with all affirmative and negative covenants associated with the Amended and Restated Credit and Security Agreement at June 30, 2018.

Revolving Line of Credit

In July 2017, the Company entered into a revolving credit and security agreement (the "Revolving Credit Agreement") which provides an aggregate revolving loan commitment of $40.0 million (which may be increased by an additional tranche of $20.0 million) with MidCap. Borrowings under the Revolving Credit Agreement bear interest at a rate of 3.95%, plus the one-month LIBOR. In addition to paying interest on the outstanding principal under the Revolving Credit Agreement, the Company paid $0.2 million of origination fee, which was 0.50% of the amount of the revolving loan. The Company recognized this origination fee as an other asset and it is being amortized to interest expense over the term of the line-of-credit. Additionally, the Company is liable for unused line fees, minimum balance fees, collateral fees, deferred revolving loan original fees, etc. This facility matures in July 2021.

As of June 30, 2018, the Company recorded approximately $9.5 million as current portion of long-term debt and approximately $429.9 million as long-term debt on the unaudited condensed consolidated balance sheets related to the 2024 Notes, the July 2017 Term Loan, and the Revolving Credit Agreement. The following table summarizes the Company's debt facilities for the periods indicated:

	As of June 30, 2018	As of December 31, 2017
	(in thousand)	
Par value of the 2024 Notes	570,000	570,000
Unamortized discount - equity component	(149,710)	(158,890)
Unamortized discount - debt issuance costs	(9,847)	(10,450)
Net carrying value of convertible debt	410,443	400,660
Other debt facilities	28,996	30,391
Net carrying value of total debt facilities	439,439	431,051

For the three months and six months ended June 30, 2018, the Company recorded $8.1 million and $15.8 million in interest expense, respectively. For the three months and six months ended June 30, 2017, the Company recorded $0.3 million and $0.5 in interest expense, respectively.

11. RESTRUCTURING

In March 2016, the Company announced a long-term plan to consolidate all of the Company's operations to Massachusetts as part of a strategic plan to increase operational efficiency. As part of the consolidation, research activities and some employees transitioned to the Company's facilities in Andover and Cambridge, Massachusetts. As of December 31, 2017, the relocations and terminations were completed.

The second floor and the first floor of the Corvallis facility were vacated and closed and made available for sub-leasing in December 2016 and April 2017, respectively. Using a discounted cash flow methodology and based on monthly rent payments as well as estimated sublease income, the Company recognized a total of approximately $1.5 million and $2.3 million in restructuring expenses for the second and the first floor, respectively. During the three month period ended June 30, 2018, the Corvallis facility was sold, and the Company entered into a rental termination agreement with the new landlord regarding the space made available for sub-lease. As a result, we relieved the remaining $2.2 million of cease-use liability related to this space in June 2018, which was recorded as a reduction to selling, general and administrative expenses.

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

	As of June 30, 2018	As of December 31, 2017
	(in thousands)	
Restructuring reserve beginning balance	$ 2,933	$ 1,588
Restructuring expenses incurred during the period	—	3,020
Amounts paid during the period	(711)	(1,675)
Reversal of cease-use liability	(2,222)	—
Restructuring reserve ending balance	$ —	$ 2,933

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,			
	2018		2017		2018		2017	
	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	528,096	$ 45.45	3,579,467	$ 13.64	1,652,681	$ 37.71	4,456,959	$ 14.16
Restricted stock units	18,695	$ 95.38	20,000	$ 36.26	169,420	$ 74.09	181,029	$ 33.03
Restricted stock awards	—	$ —	335,000	$ 34.65	17,090	$ 71.45	341,500	$ 34.58

Stock-based Compensation Expense

For the three months ended June 30, 2018 and 2017, total stock-based compensation expense was $15.3 million and $10.5 million, respectively. For the six months ended June 30, 2018 and 2017, total stock-based compensation expense was $25.8 million and $16.2 million, respectively. The increase in stock-based compensation expense for both the three and the six months was partially driven by the achievement of a milestone related to the September 2016 restricted stock awards with performance conditions. 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,	
	2018	2017	2018	2017
	(in thousands)			
Research and development	$ 5,029	$ 2,195	$ 7,089	$ 4,069
Selling, general and administrative	10,250	8,270	18,716	12,108
Total stock-based compensation expense	$ 15,279	$ 10,465	$ 25,805	$ 16,177

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,	
	2018	2017	2018	2017
	(in thousands)			
Stock options	$ 9,382	$ 6,763	$ 18,355	$ 11,801
Restricted stock awards/units	5,407	3,250	6,667	3,454
Employee stock purchase plan	490	452	783	922
Total stock-based compensation expense	$ 15,279	$ 10,465	$ 25,805	$ 16,177

13. OTHER INCOME AND LOSS

The following table summarizes other income and loss for the periods indicated:

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2018	2017	2018	2017
	(in thousand)			
Interest expense	$ (8,145)	$ (265)	$ (15,779)	$ (523)
Interest income	1,887	189	3,759	375
Amortization of investment discount	1,569	160	2,828	260
Other (expense) income	(529)	100	(511)	407
Gain from sale of Priority Review Voucher	—	—	—	125,000
Total other (loss) income	$ (5,218)	$ 184	$ (9,703)	$ 125,519

14. INCOME TAXES

The Company's tax provision for interim periods is determined using an estimate of its annual effective tax rate, adjusted for discrete items arising in that quarter. In each quarter, the Company updates its estimate of the annual effective tax rate, and if the estimated annual tax rate changes, the Company makes a cumulative adjustment in that quarter.

Deferred tax assets and deferred tax liabilities are recognized based on temporary differences between the financial reporting and tax basis of assets and liabilities using statutory rates. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all of the deferred tax assets will not be realized. Based upon the Company's history of operating losses and the uncertainty surrounding the realization of the favorable tax attributes in future tax returns, the Company has recorded a full valuation allowance against the Company's otherwise recognizable net deferred tax assets. There was no significant income tax provision or benefit recorded for the three and six months ended June 30, 2018.

15. NET (LOSS) EARNINGS PER SHARE

Basic net (loss) earnings per share is computed by dividing net (loss) income 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. For the three months ended June 30, 2018 and 2017, as well as for the three months ended June 30, 2017, there were no differences between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive due to the net loss position and, therefore, would be excluded from the diluted net loss per share calculation.

	For the Three Months Ended June 30,		For the Six Months Ended June 30,	
	2018	2017	2018	2017
	(in thousands, except per share amounts)			
Net (loss) income	$ (109,267)	$ (63,046)	$ (144,630)	$ 21,044
Weighted-average number of shares of common stock and common stock equivalents outstanding:				
Weighted-average number of shares of common stock outstanding for computing basic (loss) earnings per share	65,484	54,976	65,060	54,913
Dilutive effect of outstanding stock awards and stock options after application of the treasury stock method*	—	—	—	1,263
Weighted-average number of shares of common stock and dilutive common stock equivalents outstanding for computing diluted loss per share	65,484	54,976	65,060	56,176
Net (loss) earnings per share - basic and diluted				
Basic (loss) earnings per share	$ (1.67)	$ (1.15)	$ (2.22)	$ 0.38
Diluted (loss) earnings per share	$ (1.67)	$ (1.15)	$ (2.22)	$ 0.37

* For the three and six months ended June 30, 2018, stock options, RSAs, RSUs, stock appreciation rights ("SAR") to purchase 9.4 million shares of the Company's common stock were excluded from the net loss per share calculation as their effect would have been anti-dilutive. For the three months ended June 30, 2017, stock options, RSAs and SARs to purchase 10.1 million shares of the Company's common stock were excluded from the net loss per share calculation as their effect would have been anti-dilutive. For the six months ended June 30, 2017, out of money stock options, unvested performance-based RSUs and RSAs whose performance milestones were not achieved and potentially issuable common stock for ESPP to purchase approximately 7.0 million shares of the Company's common stock were excluded from the net earnings per share calculation as their effect would have been anti-dilutive.

16. COMMITMENTS AND CONTINGENCIES

Lease Obligations

In April 2018, the Company entered into the seventh amendment to its Cambridge, Massachusetts headquarters lease which extended the original term of the lease to September 30, 2025 and increased the total rental space by approximately 63,698 square feet.

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

	As of June 30, 2018 (in thousands)
2018 (July - December)	$ 3,277
2019	7,343
2020	7,401
2021	8,574
2022	8,862
Thereafter	25,328
Total minimum lease payments	$ 60,785

Manufacturing Obligations

The Company has entered into long-term contractual arrangements from time to time for the provision of goods and services. In addition to contract manufacturing agreements already in place, in June 2018, the Company entered into the Brammer Manufacturing Agreement with Brammer. Please see *Note 3, Collaboration, License, and Manufacturing Agreements*, for further information on this agreement.

The following table summarizes the aggregate non-cancelable contractual obligations arising from our manufacturing obligations:

	As of June 30, 2018 (in thousands)
2018 (July - December)	$ 68,824
2019	64,676
2020	45,939
2021	32,000
2022	40,000
Thereafter	160,000
Total manufacturing commitments	$ 411,439

Litigation

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

about January 15, 2016, was material and would have changed the Court's ruling. On February 26, 2016, the First Circuit stayed the appeal pending the district court's ruling on the 60(b)(2) motion. Defendants opposed the 60(b)(2) motion, and on April 21, 2016, the Court denied Plaintiffs' motion for relief from judgment. On May 19, 2016, Plaintiffs filed a motion to alter or amend the April 21, 2016 order pursuant to Federal Rule of Civil Procedure 59(e). On May 20, 2016, the Court denied Plaintiffs' motion, and Plaintiffs filed a notice of appeal of the Court's April 21, 2016 denial of their 60(b)(2) motion and May 20, 2016 denial of their 59(e) motion. On June 13, 2016, the First Circuit granted Plaintiffs' motion to consolidate the two appeals. Oral argument took place on March 7, 2017 and the First Circuit affirmed the District Court's dismissal of this case on August 22, 2017. Plaintiffs filed a Petition for Panel Rehearing and Rehearing *En Banc*, which the First Circuit denied on October 11, 2017. The period for filing a petition with the U.S. Supreme Court for a writ of certiorari has elapsed without a filing from the plaintiffs. As such, there is no risk of loss in connection with this litigation.

Another complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 styled William Kader, Individually and on Behalf of All Others Similarly Situated v. Sarepta Therapeutics Inc., Christopher Garabedian, and Sandesh Mahatme (*Kader v. Sarepta et.al 1:14-cv-14318*). On March 20, 2015, Plaintiffs filed an amended complaint asserting violations of Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5 against the Company, and Chris Garabedian and Sandy Mahatme ("Individual Defendants," and collectively with the Company, the "Kader Defendants"), and violations of Section 20(a) of the Exchange Act against the Individual Defendants. Plaintiffs alleged that the Kader Defendants made material misrepresentations or omissions during the putative class period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company's data for submission of an NDA for eteplirsen and the likelihood of the FDA accepting the NDA based on that data. Plaintiffs sought compensatory damages and fees. The Kader Defendants moved to dismiss the amended complaint on May 11, 2015. On April 5, 2016, following oral argument on March 29, 2016, the Court granted Defendants' motion to dismiss. On April 8, 2016, Lead Plaintiffs filed a motion for leave to file an amended complaint, which Defendants opposed. On January 6, 2017, the Court denied Plaintiffs' motion for leave to amend and dismissed the case. Plaintiffs filed a notice of appeal on February 3, 2017. Oral argument took place on December 4, 2017 and the First Circuit affirmed the District Court's dismissal of this case on April 4, 2018. The period for filing a petition with the U.S. Supreme Court for a writ of certiorari has elapsed without a filing from the plaintiffs. As such, there is no risk of loss in connection with this litigation.

On February 5, 2015, a derivative suit was filed in the 215th Judicial District of Harris County, Texas against the Company's Board of Directors (*David Smith, derivatively on behalf of Sarepta Therapeutics, Inc., v. Christopher Garabedian et al., No. 2015-06645*). The claims alleged that Sarepta's directors caused Sarepta to disseminate materially false and/or misleading statements in connection with disclosures concerning the Company's submission of the NDA for eteplirsen. Plaintiff sought unspecified compensatory damages, actions to reform and improve corporate governance and internal procedures, disgorgement of profits, benefits and other compensation obtained by the directors, and attorneys' fees. On July 10, 2018, Plaintiff filed a Notice of Nonsuit as to all causes of action asserted in the complaint. On July 11, 2018, the court accepted the Notice of Nonsuit and all causes of action asserted in the complaint were dismissed with prejudice. As such, there is no risk of loss in connection with this litigation.

On March 16, 2016, a derivative suit was filed in the U.S. District Court for the District of Massachusetts against the Company's Board of Directors (*Dawn Cherry, on behalf of nominal defendant Sarepta Therapeutics, Inc., v. Behrens et al., No. 16-cv-10531*). The claims alleged that the defendants authorized the Company to make materially false and misleading statements about the Company's business prospects in connection with its development of eteplirsen from July 10, 2013 through the date of the complaint. Plaintiffs sought unspecified damages, actions to reform and improve corporate governance and internal procedures, and attorneys' fees. On July 23, 2018, Plaintiffs filed a Notice of Voluntary Dismissal and dismissed their claims without prejudice. As such, there is no risk of loss in connection with this litigation.

Additionally, on September 23, 2014, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (*Terry McDonald, derivatively on behalf of Sarepta Therapeutics, Inc., et al. v. Goolsbee et al., No. 10157*). The claims allege, among other things, that (i) the Company's non-employee directors paid themselves excessive compensation fees for 2013, (ii) that the compensation for the Company's former Chief Executive Officer, Christopher Garabedian, was also excessive and such fees were the basis for Mr. Garabedian's not objecting to or stopping the excessive fees for the non-employee directors and (iii) that the disclosure in the 2013 proxy statement was deficient. The relief sought, among others, includes disgorgement and rescindment of allegedly excessive or unfair payments and equity grants to Mr. Garabedian and the directors, unspecified damages plus interest, a declaration that the Company's Amended and Restated 2011 Equity Plan at the 2013 annual meeting was ineffective and a revote for approved amendments, correction of misleading disclosures and plaintiff's attorney fees. The parties have agreed to a Memorandum of Understanding concerning the settlement terms. The court has preliminarily approved this settlement, and a notice of proposed settlement has been sent to shareholders. The settlement hearing is scheduled for September 4, 2018. The Company does not believe that disposition of the McDonald suit will have a material financial impact on the Company.

17. SUBSEQUENT EVENTS

In August 2018, the Company entered into a License, Development and Option Agreement (the “License Agreement”) and an Equity Investment Agreement with Lacerta Therapeutics, Inc. (“Lacerta”). The Company will in-license one of Lacerta’s preclinical programs (the “License”) and has options to in-license additional programs (the “Options”) for additional consideration. In connection with the closing of these transactions, the Company will make an equity investment of $30 million and an up-front payment of $8 million to Lacerta in consideration of the License and the Options. Under the License Agreement, the Company may be liable for additional payments relating to development and commercialization milestones and a high single digit royalty upon commercialization of the product. If the Company decides to exercise the Options to in-license additional programs, it may be liable for additional payments relating to development and commercialization milestones and tiered high single digit royalties upon commercialization of the products.

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

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, 2017 under the caption "Part II-Item 7 — Management's Discussion and Analysis of Financial Condition and Results of Operations". This discussion 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 contain projections of future results of operations or financial condition, or state other "forward-looking" information. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to:

- *our expectations regarding the continued growth of our business operations due, in part, to the commercialization of EXONDYS 51® (eteplirsen) Injection ("EXONDYS 51");*
- *our pipeline, technologies and next-generation approaches and their respective potential benefits, including the potential of our phosphorodiamidate morpholino oligomer ("PMO") based compounds to reduce off-target effects and be rapidly designed to target specific tissues, genetic sequences, or pathogens; the potential of our peptide-conjugated PMO ("PPMO") to be tailored to reach other organs beyond muscle; the potential of micro-dystrophin and GALGT2 to treat all or nearly all Duchenne muscular dystrophy ("DMD") patients regardless of mutation; and CRSPR/Cas9's potential to be used to fix stop codon mutations in the dystrophin gene so that dystrophin can be translated to a function protein;*
- *our belief that our highly differentiated, novel, proprietary and innovative RNA-targeted PMO-based platforms may represent a significant improvement over other RNA-targeted technologies;*
- *Our belief that our PMO-based compounds could potentially be applied to treat a broad spectrum of diseases;*
- *our belief that golodirsen and casimersen will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping;*
- *the timely completion and satisfactory outcome of our post-marketing requirements and commitments, including verification of a clinical benefit for EXONDYS 51 in confirmatory trials;*
- *our ability to successfully expand the global footprint of eteplirsen in jurisdictions in which we have yet to obtain or do not have any near term ability or plans to obtain a full regulatory approval, including through obtaining an approval from the European Medicines Agency in the EU ("EMA"), establishing compliant and successful managed access programs ("MAP"), expanding our MAPs to include more countries over time, entering into any additional distribution, service and other contracts and building out the commercial, medical and other company infrastructure necessary to support the launch and support the distribution of eteplirsen in jurisdictions outside of the U.S.;*
- *the expectation that the re-examination process of our marketing authorization application ("MAA") for eteplirsen be completed by the Committee for Medicinal Products for Human Use ("CHMP") by year-end 2018;*
- *the potential acceptance of EXONDYS 51, and our product candidates if they receive regulatory approval, in the marketplace and the accuracy of our projections regarding the market size in each of the jurisdictions that we target;*
- *our ability to further secure long term supply of EXONDYS 51 and our product candidates, including our PPMO, to satisfy our planned commercial, MAP, named-patient program and clinical needs;*
- *our expectations regarding our ability to successfully conduct or accelerate research, development, pre-clinical, clinical and post-approval trials, and our expectations regarding the timing, design and results of such trials, including the potential consistency of data produced by these trials with prior results, as well as any new data and analyses relating to the safety profile and potential clinical benefits of EXONDYS 51 and our product candidates, including golodirsen, casimersen, PPMO and gene therapy-based product candidates;*
- *the impact of regulations and regulatory decisions and guidelines by the United States Food and Drug Administration ("FDA") and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations;*
- *the plan to submit to the FDA an action plan that will include the use of GMP-s plasmid for Nationwide Children's Hospital's Phase 1/2a DMD micro-dystrophin gene therapy trial, and our expectation that, subject to the FDA's acceptance of such action plan and the release of the clinical hold on this trial, there will be no material delay in dosing patients as originally planned by year-end 2018.*

- *the possible impact of any competing products on the commercial success of EXONDYS 51 and our product candidates and our ability to compete against such products;*
- *our expectation that private insurers will continue to consider the efficacy, cost-effectiveness and safety of EXONDYS 51, in determining whether to approve reimbursement for EXONDYS 51 and at what levels;*
- *our ability to enter into research, development or commercialization alliances with universities, hospitals, independent research centers, non-profit organizations, pharmaceutical and biotechnology companies and other entities for specific molecular targets or selected disease indications and our ability to selectively pursue opportunities to access certain intellectual property rights that complement our internal portfolio through license agreements or other arrangements;*
- *our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future;*
- *the extent of protection that our patents provide and our pending patent applications may provide, if patents issue from such applications, to our technologies and programs, and our ability to obtain and maintain patent protection for our technologies and programs;*
- *our plans and ability to file and progress to issue additional patent applications to enhance and protect our new and existing technologies and programs;*
- *our ability to invalidate some or all of the claims of patents issued to competitors and pending patent applications if issued to competitors, and the potential impact of those claims on the potential commercialization and continued commercialization, where authorized, of EXONDYS 51 and the potential commercialization of our product candidates, including golodirsen, casimersen, PPMO and gene therapy-based product candidates;*
- *our ability to operate our business without infringing the intellectual property rights of others;*
- *our intention to expand our insurance coverage to include the sale of commercial products in connection with the FDA's approval of EXONDYS 51;*
- *our belief that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve months and statements about our future capital needs;*
- *our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;*
- *our ability to raise additional funds to support our business plans and strategies, including business development, and the impact of our amended and restated credit and security agreement with MidCap Financial Trust, a Delaware statutory trust ("MidCap"), as administrative agent and new revolving credit and security agreement with MidCap, on our financial condition and future operations;*
- *Expected milestones and payments in connection with our agreement with Myonexus Therapeutics, Inc.("Myonexus"), and Myonexus' expectation to initiate a Phase 1/2a trial for its MYO-101 program in the third quarter of 2018;*
- *our expectations relating to potential funding from government and other sources for the development of some of our product candidates;*
- *our ability to attract and retain key employees needed to execute our business plans and strategies and our expectations regarding our ability to manage the impact of any loss of key employees;*
- *our ability to comply with applicable environmental laws and regulations;*
- *the impact of the potential achievement of performance conditions and milestones relating to our stock awards; and*
- *our beliefs and expectations regarding milestone, royalty or other payments that could be due to third parties under existing agreements.*

We undertake no obligation to update any of the forward-looking statements contained in this 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 ("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 medicine approaches for the treatment of rare neuromuscular diseases. Applying our proprietary, highly-differentiated and innovative RNA-targeted platform technologies, we are able to develop candidate therapies for a broad range of diseases and disorders.

Our first commercial product in the U.S., EXONDYS 51, was granted accelerated approval by the FDA on September 19, 2016. EXONDYS 51 is indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

A summary description of our product and main product candidates is as follows:

- *EXONDYS 51*, our first product, uses our PMO chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. EXONDYS 51 is designed to bind to exon 51 of dystrophin pre-messenger RNA ("mRNA"), resulting in exclusion, or "skipping", 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.

  We are in the process of conducting, starting or planning various EXONDYS 51 clinical trials, including studies that are required to comply with regulatory new drug application ("NDA") and/or MAA filing requirements as well as studies we need to conduct to comply with our post-marketing FDA requirements/commitments to verify and describe clinical benefit of EXONDYS 51.

- *Golodirsen*, one of our main product candidates, uses our PMO chemistry and exon-skipping technology to skip exon 53 of the DMD gene. Golodirsen is designed to bind to exon 53 of dystrophin pre-mRNA, resulting in exclusion, or "skipping", of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein.

  We are enrolling and dosing patients in ESSENCE (Study 4045-301), our Phase 3 placebo controlled confirmatory trial in patients who have a confirmed mutation of the DMD gene that is amenable to exon 45 or 53 skipping using casimersen and golodirsen, respectively. Golodirsen is currently also in the clinic as part of a Phase 1/2 study. Part I has been completed, and Part II, an open-label portion of this study, is ongoing (Study 4053-101). In September 2017, we announced positive results of an analysis that included biopsies of the bicep muscle at baseline and on-treatment at the Part II, Week 48 time point. The study results demonstrated statistical significance on all primary and secondary biological endpoints. On March 12, 2018, we announced our plan to submit an NDA to the FDA by year-end 2018 for accelerated approval of golodirsen (SRP-4053) in patients with DMD who are amenable to skipping exon 53. Golodirsen will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping.

- *Casimersen*, one of our main product candidates, uses our PMO chemistry and exon-skipping technology to skip exon 45 of the DMD gene. Casimersen is designed to bind to exon 45 of dystrophin pre-mRNA, resulting in exclusion, or "skipping", of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 45 skipping. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein.

  We are enrolling and dosing patients in ESSENCE, further described above. Pursuant to an ongoing Sarepta-sponsored Phase 1/2 clinical trial studying casimersen (Study 4045-101), we have completed a dose titration portion (Phase 1) and are currently conducting the open-label portion of the study (Phase 2). Casimersen will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping.

- *SRP-5051*, one of our main product candidates, uses our next-generation chemistry platform, 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, or "skipping", 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 received clearance from the FDA and commenced a first-in-human, single ascending dose, study for our PPMO for the treatment of DMD in patients who are amenable to exon 51 skipping (SRP-5051). We expect to have data regarding safety and future dosing for SRP-5051 in the second half of 2018. In addition to SRP-5051, our 2018 plans currently include IND-enabling pre-clinical work on 5 additional PPMOs.

In addition to advancing our exon-skipping product candidates for DMD, we are working with several strategic partners under various agreements to research and develop multiple treatment approaches to DMD. These strategic partners include:

- Nationwide Children's Hospital, with whom we are collaborating on the advancement of (1) their micro-dystrophin gene therapy program under a research and exclusive license option agreement and (2) their Galgt2 gene therapy program under an exclusive license agreement. In the fourth quarter of 2017, the IND applications for both of these programs were cleared by the FDA, and two Phase 1/2a clinical trials in individuals with DMD were initiated. On June 19, 2018, Nationwide Children's Hospital presented positive preliminary results from its Phase 1/2a micro-dystrophin gene therapy clinical trial in the first three individuals with DMD enrolled in the trial.
- Myonexus, which develops gene therapy programs for various forms of Limb-girdle muscular dystrophies ("LGMDs"). On May 3, 2018, we entered into an agreement with Myonexus that provides us with an exclusive option to acquire Myonexus by making an option exercise payment to Myonexus plus contingent payments, if earned.
- Genethon, with whom we are collaborating on the advancement of their micro-dystrophin gene therapy program under a sponsored research and exclusive license option agreement.
- Duke University, with whom we are collaborating on the advancement of gene editing CRISPR/Cas9 technology for muscular dystrophy under a sponsored research and exclusive license option agreement that grants us rights to certain of Duke University's intellectual property for CRISPR/Cas9.
- Summit (Oxford) Ltd. ("Summit"), with whom we are collaborating under an exclusive License and Collaboration Agreement that grants us exclusive rights to Summit's utrophin modulator pipeline, including ezutromid, in Europe, Turkey and the Commonwealth of Independent States and an option to acquire rights in Latin America. On June 27, 2018, Summit announced that it has decided to discontinue the development of ezutromid after reviewing the top-line results from its Phase 2 trial.

Our Proprietary Platform Technologies

Our RNA-targeted technologies work at the most fundamental level of biology and potentially could have a meaningful impact across a broad range of human diseases and disorders. Our lead program focuses on the development of disease-modifying therapeutic candidates for DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. The basis of our novel RNA-targeted therapeutics is the PMO.

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

Our next generation PMO-based chemistries include PPMO, PMO-X® and PMO*plus*®. PPMO features covalent attachment of a cell-penetrating peptide to a PMO with the goal of enhanced delivery into the cell. In pre-clinical research, our proprietary class of PPMO compounds demonstrated an increase in dystrophin production and a more durable response compared to PMO. In addition, PPMO treatment in non-human primates is well tolerated and results in high levels of exon-skipping in skeletal, cardiac and smooth muscle tissues. Pre-clinical trials also indicate that PPMOs may require less frequent dosing than PMO, and that PPMOs could potentially be tailored to reach other organs beyond muscle.

We also collaborate with different partners to explore a gene therapy approach to DMD. The programs in collaboration with Nationwide Children's Hospital and Genethon look to express a smaller but still functional version of dystrophin ("micro-dystrophin"). Micro-dystrophin is used because normal-sized dystrophin is too large to fit in an adeno-associated virus ("AAV"). An additional program, also in collaboration with Nationwide Children's Hospital, aims to express the enzyme GALGT2 from an AAV vector. We believe that GALGT2 modifies the dystrophin associated protein complex ("DAPC") and up-regulates utrophin (a protein significantly homologous to dystrophin) to protect muscle from damage in the absence of dystrophin. The micro-dystrophin and GALGT2 technologies have the potential to treat all or nearly all DMD patients regardless of mutation.

We are also exploring, in collaboration with Duke University, the gene-editing technology CRSPR/Cas9 that aims to restore dystrophin expression by removing or "excising" exons directly from the dystrophin gene to correct out-of-frame mutations. CRSPR/Cas9 technology can also potentially be used to fix stop codon mutations in the dystrophin gene so that dystrophin can be translated to a function protein.

Manufacturing

We have developed proprietary state-of-the-art Chemistry, Manufacturing and Controls ("CMC") and manufacturing capabilities that allow synthesis and purification of our product candidates to support both clinical development as well as commercialization. Our current main focus in manufacturing is to continue scaling up production of our PMO-based therapies and optimizing manufacturing for PPMO and gene therapy-based product candidates. 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. In 2017, we opened a facility in Andover, Massachusetts, which significantly enhances our research and development manufacturing capabilities. However, we currently do not have internal large scale Good Manufacturing Practices ("GMP") manufacturing capabilities to produce our product and product candidates for commercial and/or clinical use.

Cash, Cash Equivalents and Investments

As of June 30, 2018, we had approximately $950.2 million of cash, cash equivalents and investments, consisting of $410.4 million of cash and cash equivalents, $538.8 of short term investments, and $1.0 million of long-term restricted investment. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve months.

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

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 United States 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 the most critical to the judgements and estimates used in the preparation of our unaudited condensed consolidated financial statements:

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

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, 2017.

Results of Operations for the Three and Six Months Ended June 30, 2018 and 2017

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

	For the Three Months Ended June 30,			
	2018	2017	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$ 73,529	$ 35,011	$ 38,518	110%
Total revenues	73,529	35,011	38,518	110%
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	6,735	506	6,229	NM
Research and development	122,848	58,908	63,940	109%
Selling, general and administrative	47,156	36,069	11,087	31%
EXONDYS 51 litigation and license charges	—	2,839	(2,839)	(100)%
Amortization of in-licensed rights	217	28	189	NM
Total cost and expenses	176,956	98,350	78,606	80%
Operating loss	(103,427)	(63,339)	(40,088)	63%
Other (loss) income:				
Interest (expense) income and other, net	(5,218)	184	(5,402)	NM
Loss before income tax expense (benefit)	(108,645)	(63,155)	(45,490)	72%
Income tax expense (benefit)	622	(109)	731	NM
Net loss	(109,267)	$ (63,046)	$ (46,221)	73%
Net loss per share - basic and diluted	$ (1.67)	$ (1.15)	$ (0.52)	46%

	For the Six Months Ended June 30,			
	2018	2017	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$ 138,133	$ 51,353	$ 86,780	169%
Total revenues	138,133	51,353	86,780	169%
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	12,317	729	11,588	NM
Research and development	169,052	88,027	81,025	92%
Selling, general and administrative	90,497	62,285	28,212	45%
EXONDYS 51 litigation and license charges	—	2,839	(2,839)	(100)%
Amortization of in-licensed rights	433	57	376	NM
Total cost and expenses	272,299	153,937	118,362	77%
Operating loss	(134,166)	(102,584)	(31,582)	31%
Other (loss) income:				
Gain from sale of Priority Review Voucher	—	125,000	(125,000)	(100)%
Interest (expense) income and other, net	(9,703)	519	(10,222)	NM
(Loss) income before income tax expense	(143,869)	22,935	(166,804)	NM
Income tax expense	761	1,891	(1,130)	(60)%
Net (loss) income	(144,630)	$ 21,044	$ (165,674)	NM
Basic (loss) earnings per share	$ (2.22)	$ 0.38	$ (2.60)	NM
Diluted (loss) earnings per share	$ (2.22)	$ 0.37	$ (2.59)	NM

* NM = Not Meaningful

Revenues

We record product revenues net of applicable discounts and allowances which include Medicaid rebates, governmental chargebacks including Public Health Services chargebacks, prompt pay discounts, co-pay assistance and distribution fees. Reserves established for these discounts and allowances are classified as reductions of accounts receivable (if no payments are required of us) or a liability (if a payment is required of us). These reserves are based on estimates of the amounts earned or to be claimed on the related sales. Our estimates take into consideration current contractual and statutory requirements. Actual amounts may ultimately differ from our estimates. If actual results are different from our estimates, we adjust these estimates, which will have an effect on earnings in the period of adjustment. Net product revenues for EXONDYS 51 for the three and six months ended June 30, 2018 increased by $38.5 million and $86.8 million compared with the three and six months ended June 30, 2017, respectively. These increases for both the three and six months ended June 30, 2018 primarily reflect increasing demand for EXONDYS 51 in the U.S.

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

Our cost of sales (excluding amortization of in-licensed rights) primarily consists of royalty payments to BioMarin Pharmaceutical, Inc. ("BioMarin") as a result of the execution of the settlement and licenses agreements in July 2017, inventory costs that relate to sales of EXONDYS 51 following our commercial launch in the U.S., and overhead costs. Prior to receiving regulatory approval for EXONDYS 51 from the FDA in September 2016, we expensed such manufacturing and material costs as research and development expenses. For EXONDYS 51 sold in the three and six months ended June 30, 2018 and 2017, the majority of related manufacturing costs incurred had previously been expensed as research and development expenses, as such costs were incurred prior to the FDA approval of EXONDYS 51. If product related costs had not previously been expensed as research and development expenses prior to receiving FDA approval, the incremental cost to produce the EXONDYS 51 sold would have been approximately $7.3 million and $2.9 million for the six months ended June 30, 2018 and 2017, respectively.

The following table summarizes the components of our cost of sales for the periods indicated:

	For the Three Months Ended June 30,			
	2018	2017	Change	Change
	(in thousands)		$	%
Royalty payments to BioMarin	$ 3,695	$ —	3,695	NM
Inventory costs related to EXONDYS 51 sold	2,019	95	1,924	NM
Overhead costs	1,019	400	619	155%
Other inventory costs	2	11	(9)	(82)%
Total cost of sales	$ 6,735	$ 506	6,229	NM

	For the Six Months Ended June 30,			
	2018	2017	Change	Change
	(in thousands)		$	%
Royalty payments to BioMarin	$ 6,812	$ —	6,812	NM
Inventory costs related to EXONDYS 51 sold	3,539	103	3,436	NM
Overhead costs	1,962	597	1,365	229%
Other inventory costs	4	29	(25)	(86)%
Total cost of sales	$ 12,317	$ 729	11,588	NM

* NM = Not Meaningful

The cost of sales for the three and six months ended June 30, 2018, increased by $6.2 million and $11.6 million compared with the same periods in 2017. The increase for both periods primarily reflects royalty payments to BioMarin as a result of the execution of the settlement and license agreements with BioMarin in July 2017 as well as increasing demand for EXONDYS 51.

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 which have not reached technological feasibility and do not have an alternative future use, and other external services, such as data management and statistical analysis support, and materials and supplies used in support of clinical programs. Indirect costs of our clinical programs include salaries, stock-based compensation and allocation of our facility costs.

Future research and development expenses may increase as our internal projects, such as those for our DMD product candidates, enter or proceed through later stage clinical development. However, our research and development efforts may not result in any approved products. Product candidates that appear promising at early stages of development may not reach the market for a variety of reasons. Similarly, any of our product candidates may be found to be unsafe or ineffective during clinical trials, may have clinical trials that take longer to complete than anticipated, may fail to receive necessary regulatory approvals, or may prove impracticable to manufacture in commercial quantities at reasonable cost and with acceptable quality.

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

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

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

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

	For the Three Months Ended June 30,		Change	Change
	2018	2017	$	%
	(in thousands)			
Up-front and milestone payments	$ 60,000	$ 22,000	$ 38,000	NM
Eteplirsen (exon 51)	11,295	10,554	741	7%
Casimersen (exon 45)	9,142	4,905	4,237	86%
Golodirsen (exon 53)	5,900	4,718	1,182	25%
PPMO platform	5,072	2,452	2,620	100%
Collaboration cost-sharing	2,865	—	2,865	NM
Other projects	1,269	854	415	49%
Internal research and development expenses	27,305	13,425	13,880	103%
Total research and development expenses	$ 122,848	$ 58,908	$ 63,940	109%

	For the Six Months Ended June 30,		Change	Change
	2018	2017	$	%
	(in thousands)			
Up-front and milestone payments	$ 60,000	$ 22,000	$ 38,000	NM
Eteplirsen (exon 51)	16,223	19,484	(3,261)	(17)%
Casimersen (exon 45)	15,338	7,987	7,351	92%
Golodirsen (exon 53)	14,405	8,140	6,265	77%
PPMO platform	8,796	3,705	5,091	100%
Collaboration cost-sharing	6,062	—	6,062	28%
Other projects	1,404	1,048	356	NM
Internal research and development expenses	46,824	25,663	21,161	82%
Total research and development expenses	$ 169,052	$ 88,027	$ 81,025	92%

The Company has revised the presentation as well as certain captions in the research and development expenses by project table presented above. “PPMO platform” of $2.5 million and $3.7 million for the three and six months ended June 30, 2017 was reclassified from “other projects” and presented separately in the table to conform to current year presentation.

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

	For the Three Months Ended June 30,			
	2018	2017	Change	Change
	(in thousands)		$	%
Up-front and milestone payments	$ 60,000	$ 22,000	38,000	NM
Clinical and manufacturing expenses	27,712	19,907	7,805	39%
Compensation and other personnel expenses	12,061	6,095	5,966	98%
Professional services	3,909	2,305	1,604	70%
Pre-clinical expenses	5,000	2,498	2,502	100%
Stock-based compensation	5,029	2,195	2,834	129%
Facility-related expenses	3,579	2,159	1,420	66%
Collaboration cost-sharing	2,865	—	2,865	NM
Research and other	2,693	1,749	944	54%
Total research and development expenses	$ 122,848	$ 58,908	$ 63,940	109%

Research and development expenses for the three months ended June 30, 2018 increased by $63.9 million, or 109%, compared with the three months ended June 30, 2017. The increase was primarily driven by the following:

- $38.0 million increase in up-front and milestone payments. We made an up-front payment of $60.0 million to Myonexus upon execution of the warrant to purchase common stock agreement in May 2018. In May 2017, we made a milestone payment of $22.0 million to Summit as the milestone of the last patient dosed in the safety arm cohort to the PhaseOut DMD study was achieved;
- $7.8 million increase in clinical and manufacturing expenses primarily due to increased patient enrollment in our ongoing ESSENCE trial as well as a ramp-up of manufacturing activities for golodirsen, casimersen and our PPMO platform. These increases were partially offset by a ramp-down of clinical trials in eteplirsen primarily because the PROMOVI trial has been fully enrolled;
- $6.0 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $1.6 million increase in professional services primarily due to accelerated company growth as a result of expansion of our research and development pipeline;
- $2.5 million increase in pre-clinical expenses primarily due to the continuing ramp-up of toxicology studies in our PPMO platform as well as golodirsen and casimersen;
- $2.8 million in stock-based compensation expense primarily driven by increase in headcount as well as achievement of a milestone related to the September 2016 restricted stock awards with performance conditions;
- $1.4 million increase in facility-related expenses due to our continuing expansion efforts; and
- $2.9 million increase in collaboration cost sharing with Summit on its Utrophin platform.

	For the Six Months Ended June 30,			
	2018	2017	Change	Change
	(in thousands)		$	%
Up-front and milestone payments	$ 60,000	$ 22,000	$ 38,000	NM
Clinical and manufacturing expenses	46,476	34,269	12,207	36%
Compensation and other personnel expenses	20,410	11,781	8,629	73%
Professional services	8,481	4,467	4,014	90%
Pre-clinical expenses	8,082	4,022	4,060	101%
Stock-based compensation	7,089	4,069	3,020	74%
Facility-related expenses	6,440	4,367	2,073	47%
Collaboration cost-sharing	6,062	—	6,062	NM
Research and other	6,012	3,052	2,960	97%
Total research and development expenses	$ 169,052	$ 88,027	$ 81,025	92%

Research and development expenses for the six months ended June 30, 2018 increased by $81.0 million, or 92%, compared with the six months ended June 30, 2017. The increase was primarily driven by the following:

- $38.0 million increase in up-front and milestone payments. We made an up-front payment of $60.0 million to Myonexus upon execution of the warrant to purchase common stock agreement in May 2018. In May 2017, we made a milestone payment of $22.0 million to Summit as the milestone of the last patient dosed in the safety arm cohort to the PhaseOut DMD study was achieved;
- $12.2 million increase in clinical and manufacturing expenses primarily due to increased patient enrollment in our ongoing ESSENCE trial as well as a ramp-up of manufacturing activities for golodirsen, casimersen and our PPMO platform. These increases were partially offset by a ramp-down of clinical trials in eteplirsen primarily because the PROMOVI trial has been fully enrolled;
- $8.6 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $4.0 million increase in professional services primarily due to accelerated company growth as a result of expansion of our research and development pipeline;
- $4.1 million increase in pre-clinical expenses primarily due to the continuing ramp-up of toxicology studies in our PPMO platform as well as golodirsen and casimersen;
- $3.0 million increase in stock-based compensation expense primarily driven by increase in headcount as well as achievement of a milestone related to the September 2016 restricted stock awards with performance conditions;
- $2.1 million increase in facility-related expenses due to our continuing expansion efforts;
- $6.1 million increase in collaboration cost sharing with Summit on its Utrophin platform; and
- $1.4 million increase in sponsored research with institutions such as Duke University and Genethon.

Selling, General and Administrative Expenses

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

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

	For the Three Months Ended June 30,			
	2018	2017	Change	Change
	(in thousands)		$	%
Professional services	$ 16,755	11,188	$ 5,567	50%
Compensation and other personnel expenses	17,061	8,735	8,326	95%
Stock-based compensation	10,250	6,070	4,180	69%
Facility-related expenses	2,285	1,404	881	63%
Former CEO severance	—	3,400	(3,400)	(100)%
Restructuring expenses	(2,222)	2,420	(4,642)	(192)%
Other	3,027	2,852	175	6%
Total selling, general and administrative expenses	$ 47,156	$ 36,069	$ 11,087	31%

Selling, general and administrative expenses for the three months ended June 30, 2018 increased by $11.1 million, or 31%, compared with the three months ended June 30, 2017. This was primarily driven by the following:

- $5.6 million increase in professional services primarily due to continuing global expansion;
- $8.3 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $4.2 million increase in stock-based compensation primarily due to an increase in headcount and the achievement of a milestone related to the September 2016 restricted stock awards with performance conditions;
- $3.4 million decrease in severance expense as a result of the termination of our former CEO in June 2017; and
- $4.6 million decrease in restructuring expenses due to the relief of cease-use liabilities as a result of the termination of the rental agreement for our Corvallis facility.

	For the Six Months Ended June 30,			
	2018	2017	Change	Change
	(in thousands)		$	%
Professional services	$ 32,909	$ 20,929	$ 11,980	57%
Compensation and other personnel expenses	31,156	17,514	13,642	78%
Stock-based compensation	18,716	9,908	8,808	89%
Facility-related expenses	4,326	3,152	1,174	37%
Former CEO severance	—	3,400	(3,400)	(100)%
Restructuring expenses	(2,222)	2,586	(4,808)	(186)%
Other	5,612	4,796	816	17%
Total selling, general and administrative expenses	$ 90,497	$ 62,285	$ 28,212	45%

Selling, general and administrative expenses for the six months ended June 30, 2018 increased by $28.2 million, or 45%, compared with the six months ended June 30, 2017. This was primarily driven by the following:

- $12.0 million increase in professional services primarily due to continuing global expansion;
- $13.6 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $8.8 million increase in stock-based compensation primarily due to an increase in headcount and the achievement of a milestone related to the September 2016 restricted stock awards with performance conditions as well as the impact of revised forfeiture rate assumption for officers and members of our Board of Directors;

- $1.2 million increase in facility-related expense primarily due to continuing global expansion;
- $3.4 million decrease in severance expense as a result of the termination of our former CEO in June 2017; and
- $4.8 million decrease in restructuring expenses due to the relief of cease-use liabilities as a result of the termination of the rental agreement for our Corvallis facility.

EXONDYS 51 litigation and license charges

As a result of the execution of the settlement and license agreements with BioMarin in July 2017, we recognized EXONDYS 51 litigation and license charges of $2.8 million which related to estimated royalties between September 2016 and June 2017. There was no such a transaction in 2018.

Amortization of In-licensed Rights

Amortization of in-license rights relate to the two agreements we entered into with BioMarin and University of Western Australia ("UWA") in July 2017 and April 2011, respectively. We recorded an in-licensed right asset of $6.6 million as a result of a settlement and license agreement with BioMarin. Additionally, following the first sale of EXONDYS 51 in September 2016, we recorded an in-licensed right asset of $1.0 million related to a license agreement with UWA. Both in-licensed rights are being amortized on a straight-line basis over the life of the patent from the first commercial sale of EXONDYS 51. For the three and six months ended June 30, 2018, we recorded amortization of in-licensed rights of approximately $0.2 million and less than $0.4 million, respectively. For both the three and six months ended June 30, 2017, we recorded amortization of in-licensed rights of less than $0.1 million.

Gain from Sale of Priority Review Voucher

In February 2017, we entered into an agreement with Gilead Sciences, Inc. ("Gilead") to sell our Rare Pediatric Disease Priority Review Voucher ("PRV"). We received the PRV when EXONDYS 51 was approved by the FDA for the treatment of patients with DMD amenable to exon 51 skipping. Following the early termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, in March 2017, we completed our sale of the PRV to a subsidiary of Gilead. Pursuant to the agreement, the subsidiary of Gilead paid us $125.0 million, which was recorded as a gain from sale of the PRV as it did not have a carrying value at the time of the sale.

Interest (expense) income and other, net

Interest (expense) income and other, net, primarily consists of interest income on our cash, cash equivalents and investments, interest expense on our debt facilities and rental income. Our cash equivalents and investments consist of money market funds, commercial paper, government and government agency debt securities and certificates of deposit. Interest expense includes interest accrued on our convertible notes, term loan, and revolving line of credit. Rental income was primarily comprised of leasing excess space in some of our facilities.

For the three and six months ended June 30, 2018, interest expense and other, net was approximately $5.2 million and $9.7 million, respectively. For the three and six months ended June 30, 2017, interest income and other, net was approximately $0.2 million and $0.5 million. The unfavorable changes primarily reflected the interest expense accrued on our debt facilities entered into during the latter half of 2017 partially offset by interest income from higher balances of cash, cash equivalents and investments.

Income tax expense (benefit)

Income tax expense for the three and six months ended June 30, 2018 was approximately $0.6 million and $0.8 million, respectively, which related to state taxes. Income tax (benefit) expense for the three and six months ended June 30, 2017 was approximately ($0.1) million and $1.9 million, respectively, which related to the alternative minimum tax related the gain from the sale of the PRV.

Liquidity and Capital Resources

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

	As of June 30, 2018	As of December 31, 2017	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 410,375	$ 599,691	$ (189,316)	(32)%
Short-term investments	538,769	479,369	59,400	12%
Long-term investment	—	9,980	(9,980)	(100)%
Restricted investment	1,001	784	217	28%
Total cash, cash equivalents and investments	$ 950,145	$ 1,089,824	$ (139,679)	(13)%
Borrowings:				
Current portion of long-term debt	$ 9,514	$ 6,175	$ 3,339	54%
Long-term debt	429,925	400,641	29,284	7%
Total borrowings	$ 439,439	$ 406,816	$ 32,623	8%
Working capital				
Current assets	$ 1,139,244	$ 1,228,644	$ (89,400)	(7)%
Current liabilities	$ 104,687	88,332	16,355	19%
Total working capital	$ 1,034,557	$ 1,140,312	$ (105,755)	(9)%

For the period ended June 30, 2018, our principal source of liquidity was derived from proceeds from product sales of EXONDYS 51 and equity and debt financings. For the period ended December 31, 2017, our principal source of liquidity was derived from proceeds from the sale of the PRV, equity and debt financings and product sales of EXONDYS 51. 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.

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

- our ability to continue to generate revenues from sales of EXONDYS 51 and potential future products;
- the timing and costs associated with our global expansion;
- the timing and costs of building out our manufacturing capabilities;
- the timing of advanced payments related to our future inventory commitments;
- the timing and costs associated with our clinical trials and pre-clinical trials;
- the attainment of milestones and our obligations to make milestone payments to Myonexus, BioMarin, Summit, UWA and other institutions;
- repayment of outstanding debts; and
- the costs of filing, prosecuting, defending and enforcing patent claims and our other intellectual property rights.

Our cash requirements are expected to continue to increase as we advance our research, development and commercialization programs and we expect to seek additional financings primarily from, but not limited to, the sale and issuance of equity, debt securities, the licensing or sale of our technologies or additional government contracts. We cannot provide assurances that financing will be available when and as needed or that, if available, the financings will be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we require, this would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

Cash Flows

	For the Six Months Ended June 30,			
	2018	2017	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ (152,922)	$ (143,035)	$ (9,887)	7%
Investing activities	$ (69,352)	189,931	(259,283)	(137)%
Financing activities	$ 32,822	(968)	33,790	NM
(Decrease) increase in cash and cash equivalents	$ (189,452)	$ 45,928	$ (235,380)	(512)%

*NM = Not Meaningful

Operating Activities. Cash used in operating activities increased by $9.9 million for the six months ended June 30, 2018 compared with the six months ended June 30, 2017. This was primarily driven by an increase of $18.1 million in non-cash adjustments, favorable changes of $12.7 million in operating assets and liabilities primarily due to timing of certain payments, including a $20 million advanced payment to Brammer Bio MA, LLC, and a decrease of $40.7 million in net loss excluding the gain from sale of the PRV driven by an increase in product sales for EXONDYS 51 partially offset by increases in research and development expenses, including a $60 million up-front payment to Myonexus, and selling, general and administrative expenses.

Investing Activities. The cash used in investing activities for the six months ended June 30, 2018 was $69.4 million and the cash provided by investing activities for the six months ended June 30, 2017 was $189.9 million. The unfavorable change was driven by increases of $195.5 million in purchase of available-for-sale securities and $13.5 million in property and equipment as well as proceeds of $125.0 million from sale of the PRV and $10.7 million in maturity of restricted investment in March 2017. These were partially offset by an increase of $85.7 million from the maturity of available-for-sale securities.

Financing Activities. Cash provided by financing activities was $32.8 million for the six months ended June 30, 2018 and cash used in financing activities was $1.0 million for the six months ended June 30, 2017. This was primarily driven by increases of $30.3 million in proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program and $173.4 million in proceeds from the revolving line of credit as well as a decrease of $5.1 million in repayment of long-term debt. These were partially offset by increases of $173.7 million payments on the revolving line of credit.

Off-Balance Sheet Arrangements

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

Contractual Payment Obligations

In our continuing operations, we have entered into long-term contractual arrangements from time to time for our facilities, the provision of goods and services, and acquisition of technology access rights, among others. The following table presents contractual obligations arising from these arrangements as of June 30, 2018:

	Payment Due by Period				
	Total	Less Than 1 Year	1 - 3 Years	3 - 5 Years	More than 5 Years
			(in thousands)		
Term loan (1)	$ 33,517	$ 11,930	$ 21,587	$ —	$ —
Convertible debt (1)	624,506	8,550	17,100	17,100	581,756
Lease obligations	60,785	6,973	15,273	17,725	20,814
Manufacturing obligations (2)	411,439	99,312	96,127	76,000	140,000
Total contractual obligations and contingencies	$ 1,130,247	$ 126,765	$ 150,087	$ 110,825	$ 742,570

(1) Interest is included.

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

Milestone Obligations

For product candidates that are currently in various research and development stages, we may be obligated to make up to $254.0 million of future development, up-front royalty and commercial milestone payments associated with our collaboration and license agreements. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory or commercial milestones. Because the achievement of these milestones is not probable and payment is not required as of June 30, 2018, 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 sales milestones.

Recent Accounting Pronouncements

For additional information, please read *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* of the unaudited condensed consolidated financial statements contained in Part I, Item 1 of this report, Form 10-Q for the quarterly period ended June 30, 2018.

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, government and government agency bonds and high-grade corporate bonds with maturities of three years or less. Our cash is deposited in and invested through highly rated financial institutions in North America. As of June 30, 2018, we had approximately $950.2 million of cash, cash equivalents and investments, comprised of $410.4 million of cash and cash equivalents, $538.8 million of short-term investments and $1.0 million restricted investment. 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, 2018, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of approximately $0.2 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, 2018, under the supervision and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of our disclosure controls and procedures pursuant to paragraph (b) of Rules 13a-15 and 15d-15 under the Securities Exchange Act of 1934 (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 Chief Executive Officer and our Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of June 30, 2018, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarterly period ended June 30, 2018, there were no changes in the Company’s internal controls over financial reporting that have materially affected or are reasonably likely to materially affect the Company’s internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings

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

Item 1A. Risk Factors.

Factors That Could Affect Future Results

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

Risks Related to Our Business

We are highly dependent on the commercial success of EXONDYS 51 in the U.S.; we may not be able to meet expectations with respect to EXONDYS 51 sales or attain profitability and positive cash-flow from operations.

On September 19, 2016, the FDA granted accelerated approval for EXONDYS 51 as a therapeutic treatment for DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 is currently commercially available in the U.S. only, although it is available in certain countries outside of the U.S. on a named patient basis and through our MAP. The commercial success of EXONDYS 51 continues to depend on a number of factors, including, but not limited to:

- the effectiveness of our sales, managed markets, marketing efforts and support for EXONDYS 51;
- the consistency of any new data we collect and analyses we conduct with prior results, whether they support a favorable safety and efficacy profile of EXONDYS 51 and any potential impact on our FDA accelerated approval status and/or FDA package insert for EXONDYS 51;
- the effectiveness of our ongoing EXONDYS 51 commercialization activities, including negotiating and entering into any additional commercial, supply and distribution contracts, scaling up manufacturing and hiring any additional personnel as needed to support commercial efforts;
- our ability to comply with FDA post-marketing requirements and commitments, including through successfully conducting additional studies that confirm clinical efficacy and safety of EXONDYS 51 and acceptance of the same by the FDA and medical community since continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials;
- the occurrence of any side effects, adverse reactions or misuse, or any unfavorable publicity in these areas;
- the cost-effectiveness of EXONDYS 51 and whether we can consistently manufacture it in commercial quantities and at acceptable costs;
- the rate and consistency with which EXONDYS 51 is prescribed by physicians, which depends on physicians' views on the safety and efficacy of EXONDYS 51;
- our ability to secure and maintain adequate reimbursement for EXONDYS 51, including during 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;
- our ability to obtain and maintain patent protection for EXONDYS 51, 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 or commercialization of competing products or therapies for the treatment of DMD, or its symptoms, and the existence of competing clinical trials;
- our ability to increase awareness of the importance of genetic testing and knowing/understanding DMD mutations, and identifying and addressing procedural barriers to obtaining therapy;
- our ability to remain compliant with laws and regulations that apply to us and our commercial activities;

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

In addition, the process leading to a patient's first infusion of EXONDYS 51 may be slower for certain patients. For example, the time to first infusion may take longer if a patient chooses to put in an intravenous port, which eases access to the vein. As the launch of EXONDYS 51 continues to progress, we expect the variation among patients to decline, leading to a faster time to infusion. However, delays in the process prior to first infusion could negatively impact the sales of EXONDYS 51.

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

We may not be able to expand the global footprint of, or obtain any significant revenues, from sales of eteplirsen outside of the U.S.

Although we contracted with third party distributors to distribute eteplirsen in certain countries outside the U.S. on a named patient basis, and initiated a limited launch of an ex-U.S. eteplirsen MAP, which we plan to expand to other jurisdictions in the future, and although we continue to pursue regulatory approval of eteplirsen in certain targeted jurisdictions, such as the EU and Israel, we may not be successful in expanding access to eteplirsen nor produce any significant revenues from eteplirsen sales outside of the U.S. For example, healthcare providers in MAP jurisdictions may not be convinced that their patients can benefit from eteplirsen or may prefer to wait until such time as eteplirsen is approved by a regulatory authority in their country before prescribing eteplirsen. Even if a healthcare provider is interested in obtaining access to eteplirsen for its patient through the MAP, the patient will not be able to obtain access to eteplirsen if payment for the drug is not secured. Additionally, we may not be able to obtain regulatory approval in the jurisdictions we have targeted, such as the EU, if our product approval applications, data packages submitted to regulatory authorities, and any additional data and analyses we submit in response to requests and concerns from regulatory authorities, do not support or convince regulatory authorities of the safety and efficacy of eteplirsen. If we fail to obtain regulatory approvals, particularly for our eteplirsen MAA in the EU, our ability to make revenues from eteplirsen sales outside of the U.S. will be limited. In addition, failure to obtain approval in one country or area may affect sales under the MAP in other countries or areas. Even if we are successful in obtaining regulatory approval of eteplirsen outside of the U.S., our revenue earning capacity will depend on commercial and medical infrastructure, pricing and reimbursement negotiations and decisions with third party payors, including government payors. See *"— Even though EXONDYS 51 has been approved for marketing in the U.S., we may not receive approval to commercialize EXONDYS 51 outside of the U.S."*

EXONDYS 51 may cause undesirable side effects or have other properties that could negatively impact its U.S. approval status and/or limit its commercial potential outside of the U.S.

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

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

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

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

We currently do not have the internal ability to undertake the manufacturing process for EXONDYS 51 or our product candidates in the quantities needed to meet commercial, clinical or MAP demand for EXONDYS 51, or to conduct our research and development programs and conduct clinical trials for our product candidates, including PPMO, golodirsen, casimersen and gene therapy-based product candidates. Therefore, we rely on, and expect to continue relying on for the foreseeable future, a limited number of third parties to manufacture and supply materials (including raw materials and subunits), API and drug product, as well as to perform additional steps in the manufacturing process, such as labeling and packaging of vials and storage of EXONDYS 51 and our product candidates. There are a limited number of third parties with facilities and capabilities suited for the manufacturing process of EXONDYS 51 and our product candidates, which creates a heightened risk that we may not be able to obtain materials and APIs in the quantity and purity that we require.

For example, we have recently been notified by the Research Institute at Nationwide Children's Hospital (the "Research Institute") that they have received a letter from the FDA on July 24, 2018, stating that their Phase 1/2a DMD micro-dystrophin gene therapy trial has been placed on clinical hold due to the presence of a trace amount of DNA fragment in research-grade third-party supplied plasmid (the "Clinical Hold"). The Research Institute, working with us, has developed an action plan with immediate plans to submit for review by the FDA, which will include the use of GMP-s plasmid for the program. Subject to the FDA's acceptance of such action plan and the release of the Clinical Hold, we do not anticipate any material delay in dosing patients as originally planned by year-end 2018. However, there is no assurance that the FDA's requirements will be met and that the Clinical Hold will be lifted in the expected timeframe, or at all. If the Clinical Hold is not lifted in the expected timeframe, the development of our micro-dystrophin gene therapy product candidate may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for or successfully commercialize this product candidate.

In addition, the process for adding new manufacturing capacity can be lengthy and could cause delays in our development efforts. Any interruption of the development or operation of those facilities due to, among other reasons, events such as order delays for equipment or materials, equipment malfunction, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters such as earthquake or fire, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available EXONDYS 51, product candidates or materials.

If these third parties were to cease providing quality manufacturing and related services to us, and we are not able to engage appropriate replacements in a timely manner, our ability to manufacture EXONDYS 51 or our product candidates in sufficient quality and quantity required for our planned commercial, pre-clinical and clinical or MAP use of EXONDYS 51 would adversely affect our various product research, development and commercialization efforts.

We have, through our third party manufacturers, produced or are in the process of producing supply of our product candidates and EXONDYS 51, respectively, based on our current understanding of market demands and our anticipated needs for our research and development efforts, clinical trials, MAPs and commercial sales. In light of the limited number of third parties with the expertise to produce EXONDYS 51 and our product candidates, the lead time needed to manufacture them, and the availability of underlying materials, we may not be able to, in a timely manner or at all, establish or maintain sufficient commercial and other manufacturing arrangements on the commercially reasonable terms necessary to provide adequate supply of EXONDYS 51 and our other product candidates to meet demands that meet or exceed our projected needs. Furthermore, we may not be able to obtain the significant financial capital that may be required in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for EXONDYS 51 and our product candidates, such parties may not comply with the terms and timelines they have agreed to for various reasons, some of which may be out of their or our control, which could impact our ability to execute our business plans on expected or required timelines in connection with the commercialization of EXONDYS 51 and the continued development of our product candidates, including our follow-on exon-skipping product candidates, PPMO and gene therapy-based product candidates. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and /or substantial termination penalties, which could have a material adverse effect on our business prior to and after commercialization.

The third parties we use in the manufacturing process for EXONDYS 51 and our product candidates may fail to comply with current GMP ("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. While we work diligently with all contract manufacturers to maintain full compliance, we do not have direct control over a third party manufacturer's compliance with these regulations and requirements. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of EXONDYS 51 and our product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively. The failure to achieve and maintain compliance with cGMP and other applicable government regulations, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in product recalls, clinical holds such as the Clinical Hold described above, delayed or withheld approvals, patient injury or death. This risk is particularly heightened as we optimize manufacturing for our product candidates, including golodirsen, casimersen, and novel programs such as PPMO and gene therapy. For example, following the imposition of the Clinical Hold, the Research Institute, working with us, has developed an action plan with immediate plans to submit for review by the FDA, which will include the use of GMP-s plasmid for the Nationwide Children's Hospital's Phase 1/2a DMD micro-dystrophin gene therapy trial. If our contract manufacturers fail to adhere to applicable cGMP and other applicable government regulations, or experience manufacturing problems, we will suffer significant consequences, including product seizures or recalls, postponement or cancellation of clinical trials, loss or delay of product approval, fines and sanctions, loss of revenue, termination of the development of a product candidate, reputational damage, shipment delays, inventory shortages, inventory write-offs and other product-related charges and increased manufacturing costs. If we experience any of these results, the success of our commercialization of EXONDYS 51 and/or our development efforts for our product candidates, including golodirsen, casimersen and novel programs such as PPMO and gene therapy, could be significantly delayed, fail or otherwise be negatively impacted.

We may not be able to successfully scale up manufacturing of EXONDYS 51 or our product candidates in sufficient quality and quantity or within sufficient timelines, or be able to secure ownership of intellectual property rights developed in this process, which could negatively impact the commercial success of EXONDYS 51 and/or the development of our product candidates and next generation chemistries like PPMO and gene therapy.

We are working to increase manufacturing capacity and scale up production of some of the components of our drug products. Our focus remains on (i) achieving larger-scale manufacturing capacity for EXONDYS 51 throughout the manufacturing supply chain (ii) continuing to increase material and API production capacity to provide the anticipated amounts of drug product needed for our planned studies for our product candidates and (iii) optimizing manufacturing for our follow-on exon skipping product candidates and novel programs, including PPMO and gene therapy. We may not be able to successfully increase manufacturing capacity or scale up the production of materials, APIs and drug products, whether in collaboration with third party manufacturers or on our own, in a manner that is safe, compliant with cGMP conditions or other applicable legal or regulatory requirements, in a cost-effective manner, in a time frame required to meet our timeline for commercialization, clinical trials and other business plans, or at all. Compliance with cGMP requirements and other quality issues may arise during our efforts to increase manufacturing capacity and scale up production with our current or any new contract manufacturers. These issues may arise in connection with the underlying materials, the inherent properties of EXONDYS 51 or a product candidate, EXONDYS 51 or a product candidate in combination with other components added during the manufacturing and packaging process or during shipping and storage of the APIs or finished drug product. In addition, in order to release EXONDYS 51 for commercial use and demonstrate stability of product candidates for use in clinical trials (and any subsequent drug products for commercial use), our manufacturing processes and analytical methods must be validated in accordance with regulatory guidelines. We may not be able to successfully validate, or maintain validation of, our manufacturing processes and analytical methods or demonstrate adequate purity, stability or comparability of EXONDYS 51 or our product candidates in a timely or cost-effective manner, or at all. If we are unable to successfully validate our manufacturing processes and analytical methods or to demonstrate adequate purity, stability or comparability, the commercial availability of EXONDYS 51 and the continued development and/or regulatory approval of our product candidates, including PPMO and gene therapy-based product candidates, may be delayed or otherwise negatively impacted, which could significantly harm our business.

During work with our third party manufacturers to increase and optimize manufacturing capacity and scale up production, it is possible that they could make proprietary improvements in the manufacturing and scale-up processes for EXONDYS 51 or our product candidates, including PPMO and gene therapy-based product candidates. We may not own or be able to secure ownership of such improvements or may have to share the intellectual property rights to those improvements. Additionally, it is possible that we will need additional processes, technologies and validation studies, which could be costly and which we may not be able to develop or acquire from third parties. Any failure to secure the intellectual rights required for the manufacturing process needed for large-scale clinical trials or commercialization of EXONDYS 51 or the continued development of our product candidates, including PPMO, could cause significant delays in our business plans or otherwise negatively impact the commercialization of EXONDYS 51 or the continued development of our product candidates, including PPMO and gene therapy-based product candidates.

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

We rely on third parties to commercially distribute EXONDYS 51 to patients in the U.S. We have contracted with a third party logistics company to warehouse EXONDYS 51 and with distributors and specialty pharmacies to sell and distribute it to patients. A specialty pharmacy is a pharmacy that specializes in the dispensing of medications for complex or chronic conditions that require a high level of patient education and ongoing management.

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 EXONDYS 51. If we are unable to effectively manage the distribution process, the sales of EXONDYS 51, as well as any future products we may commercialize, could be delayed or severely compromised and our results of operations may be harmed.

In addition, the use of 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 EXONDYS 51 or serious adverse events and/or product complaints regarding EXONDYS 51;
- not effectively sell or support EXONDYS 51;
- reduce or discontinue their efforts to sell or support EXONDYS 51;
- not devote the resources necessary to sell EXONDYS 51 in the volumes and within the time frame we expect;
- be unable to satisfy financial obligations to us or others; or
- cease operations.

Any such events may result in decreased product sales, 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 eteplirsen to patients outside of the U.S., we have contracted with third party distributors and service providers to distribute eteplirsen in certain countries on a named patient basis and through our ex-U.S. MAP. We will need to continue building out our network for commercial distribution in jurisdictions in which eteplirsen is 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 eteplirsen. Any such events may result in regulatory actions that may include suspension or termination of the distribution and sale of eteplirsen in a certain country, loss of revenue, and/or reputational damage, which could harm our results of operations and business.

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

We have hired and trained a commercial team and put in the organizational infrastructure we believe we need to support the commercial success of EXONDYS 51 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 EXONDYS 51, to deliver a consistent message regarding EXONDYS 51 and be effective in convincing physicians to prescribe EXONDYS 51;
- an inability to equip sales personnel with compliant and effective materials, including medical and sales literature to help them educate physicians and our healthcare providers regarding EXONDYS 51 and its proper administration and educate payors on the safety and efficacy profile of EXONDYS 51 to support favorable coverage decisions; and
- unforeseen costs and expenses associated with maintaining and further developing an independent sales and marketing organization.

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 EXONDYS 51 in the U.S., which would adversely affect our business and financial condition.

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

Our ability to successfully maintain and/or increase EXONDYS 51 sales 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 EXONDYS 51, or we may be required to sell EXONDYS 51 at an unsatisfactory price.

We expect that private insurers will continue to consider the efficacy, cost-effectiveness and safety of EXONDYS 51, 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 EXONDYS 51 and at what levels. If any new data and information we collect is not favorable, third party insurers may make coverage decisions that negatively impact sales of EXONDYS 51. We continue to have discussions with payors, some of which may eventually deny coverage. We may not receive approval for reimbursement of EXONDYS 51 from 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 insurers, including managed care organizations, the Medicare or Medicaid programs or other reimbursing bodies or payors limit the indications for which EXONDYS 51 will be reimbursed or fail to recognize accelerated approval and surrogate endpoints as clinically meaningful.

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

In some foreign countries, particularly Canada and the countries of Europe, 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 European countries have implemented government measures to either freeze or reduce pricing of pharmaceutical products. If reimbursement for our products is unavailable in any country in which reimbursement is sought, limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed. In addition, many foreign countries are referencing to other countries' official public 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 EXONDYS 51 and our 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.

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

The U.S. government and individual states have aggressively pursued healthcare reform, as evidenced by the passing of the Healthcare Reform Act and the ongoing efforts to modify or repeal that legislation. The Healthcare Reform Act substantially changed the way healthcare is financed by both governmental and private insurers and contains a number of provisions that affect coverage and reimbursement of drug products and/or that could potentially reduce the demand for pharmaceutical products such as increasing drug rebates under state Medicaid programs for brand name prescription drugs and extending those rebates to Medicaid managed care and assessing a fee on manufacturers and importers of brand name prescription drugs reimbursed under certain government programs, including Medicare and Medicaid. Other aspects of healthcare reform, such as expanded government enforcement authority and heightened standards that could increase compliance-related costs, could also affect our business. Modifications have been implemented under the Trump Administration and additional modifications or repeal may occur. See *"GOVERNMENT REGULATION- Pharmaceutical Pricing and Reimbursement- Third Party Reimbursement and Pricing in the U.S.-Healthcare and Other Reform."* We cannot predict the ultimate content, timing or effect of any changes to the Healthcare Reform Act or other federal and state reform efforts. There is no assurance that federal or state health care reform will not adversely affect our future business and financial results, and we cannot predict how future federal or state legislative, judicial or administrative changes relating to healthcare reform will affect our business.

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, waiver from Medicaid drug rebate law requirements, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. We anticipate that the U.S. Congress, state legislatures and the private sector will continue to consider and may adopt healthcare policies intended to curb rising healthcare costs. These cost containment measures may include:

- controls on government funded reimbursement for drugs;
- caps or mandatory discounts under certain government sponsored programs;
- controls on healthcare providers;
- challenges to the pricing of drugs or limits or prohibitions on reimbursement for specific products through other means;
- reform of drug importation laws;
- 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.

We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third party coverage and reimbursement may be enacted in the future or what effect such legislation, regulations or policies would have on our business. Any cost containment measures, including those listed above, or other healthcare system reforms that are adopted, could significantly decrease the available coverage and the price we might establish for EXONDYS 51 and our other potential products, which would have an adverse effect on our net revenues and operating results.

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

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

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

Continued approval for this indication 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 EXONDYS 51; 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 EXONDYS 51 studies, would lead to negative regulatory action from the FDA and/or withdrawal of regulatory approval of EXONDYS 51, and could also negatively impact a decision from EMA on our MAA. In addition, if additional data we collect on eteplirsen in connection with our MAA does not support the safety and efficacy or EXONDYS 51, our approval status in the U.S. could be negatively impacted.

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, or suspension of manufacturing. Sponsors of drugs approved under FDA accelerated approval provisions also are required to submit to 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 EXONDYS 51 fail to comply with applicable regulatory requirements, a regulatory agency may:

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

Even though EXONDYS 51 has been approved for marketing in the U.S., we may not receive approval to commercialize EXONDYS 51 outside of the U.S.

We are not permitted to market or sell EXONDYS 51 in the EU or in any other foreign countries on a commercial basis until we receive the requisite approval from such country's regulatory authorities. In order to market any product in a foreign country, we must comply with numerous and varying regulatory requirements for approval in those countries regarding demonstration of evidence of the product's safety and efficacy and governing, among other things, labeling, distribution, advertising, and promotion, as well as pricing and reimbursement of the product. Approval procedures vary among countries, and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ significantly from that required to obtain approval in the U.S. In particular, in many foreign countries, it is required that a product receives pricing and reimbursement approval before the product can be distributed commercially. This can result in substantial delays, and the price that is ultimately approved in some countries may be lower than the price for which we expect to offer EXONDYS 51.

Marketing approval in one country does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may have a negative effect on the approval process in others. Failure to obtain marketing approval in other countries or any delay or setback in obtaining such approval would impair our ability to develop foreign markets for eteplirsen and could adversely affect our business and financial condition. Any such complications may reduce our target market and delay or limit the full commercial potential of eteplirsen. Many foreign countries are undertaking cost-containment measures that could affect pricing or reimbursement of eteplirsen.

In November 2016, we submitted an MAA for eteplirsen to the EMA and the application was validated in December 2016. As we announced on June 1, 2018, the CHMP of the EMA has recently adopted a negative opinion for eteplirsen. Although we requested a re-examination and that a Scientific Advisory Group (SAG) be called to provide expert guidance and insight into DMD, the CHMP may render a negative final decision for eteplirsen. The re-examination process is expected to be completed by year-end 2018.

Obtaining approval of an MAA or any other application for approval in a foreign country is an extensive, lengthy, expensive and uncertain process, and the regulatory authority may reject an application or delay, limit or deny approval of eteplirsen for many reasons, including:

- we may not be able to demonstrate to the satisfaction of foreign regulatory authorities that eteplirsen is safe and effective for the treatment of patients with DMD who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping;
- the results of clinical trials may not meet the level of statistical or clinical significance required for approval by foreign regulatory authorities;
- foreign regulatory authorities may disagree with the adequacy (number, design, size, controls, conduct or implementation) of our clinical trials prior to granting approval, and we may not be able to generate the required data on a timely basis, or at all;
- regulatory authorities may conclude that data we submit to them, including data from clinical trials or any other additional data and analyses we submit in support of an approval or in response to requests from regulatory authorities, fail to demonstrate an appropriate level of safety or efficacy of eteplirsen or that eteplirsen's clinical benefits outweigh its safety risks; or such regulatory authorities may disagree with our interpretation of data from pre-clinical trials or clinical trials and require that we conduct one or more additional trials;
- regulatory authorities outside the U.S. may not accept data generated at our clinical trial sites 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 eteplirsen, thus limiting intended users or providing an additional hurdle for market acceptance of the product;
- regulatory authorities outside the U.S. may identify deficiencies in the manufacturing processes, or may require us to change our manufacturing process or specifications;
- we may not be able to validate our manufacturing process to the satisfaction of regulatory authorities outside the U.S. or demonstrate adequate cGMP compliance; or
- regulatory authorities outside the U.S. may adopt new or revised approval policies and regulations.

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

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

EXONDYS 51 may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our potential profitability and future business prospects.

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

- our ability to demonstrate to the medical community, including specialists who may purchase or prescribe EXONDYS 51, the clinical efficacy and safety of EXONDYS 51 as the prescription product of choice DMD amenable to exon-51 skipping in the U.S.;
- the effectiveness of our sales and marketing organizations and distribution networks;

- the ability of patients or providers to be adequately reimbursed for EXONDYS 51 in a timely manner from government and private payors;
- the actual and perceived efficacy and safety profile of EXONDYS 51, particularly if unanticipated adverse events related to EXONDYS 51 treatment arise and create safety concerns among potential patients or prescribers or if new data and analyses we obtain for eteplirsen do not support, or are interpreted by some parties to not support, the efficacy of EXONDYS 51; and
- the efficacy and safety of our other exon-skipping product candidates, including our exon 45 and exon 53 product candidates, and third parties' competitive therapies.

The patient population suffering from DMD, and in particular those with mutations amenable to exon-51 skipping, is small and has not been established with precision. If the actual number of patients is smaller than we estimate, our revenue and ability to achieve profitability may be adversely affected.

DMD is a fatal genetic neuromuscular disorder affecting an estimated one in approximately every 3,500 to 5,000 males born worldwide, of which up to 13% are estimated to be amenable to exon-51 skipping. Our estimate of the size of the patient population is based on published studies as well as internal analyses. If the results of these studies or our analysis of them do not accurately reflect the number of patients with DMD, our assessment of the market may be inaccurate, making it difficult or impossible for us to meet our revenue goals, or to obtain and maintain profitability. The small population of DMD patients may also delay patients' recruitment for our clinical trials, especially in light of competing clinical trials.

Since EXONDYS 51 targets a small patient population, the per-patient drug pricing must be high in order to recover our development and manufacturing costs, fund adequate patient support programs, fund additional research and achieve profitability. We may be unable to maintain or obtain sufficient sales volumes at a price high enough to justify our product development efforts and our sales, marketing and manufacturing expenses.

We have been granted orphan drug exclusivity for EXONDYS 51 in the U.S. and an orphan drug designation for eteplirsen in the EU, however, there can be no guarantee that we will be able to maintain orphan exclusivity for such product and product candidates nor that we will receive orphan drug approval or exclusivity and prevent third parties from developing and commercializing products that are competitive to EXONDYS 51 or our other product candidates.

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

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

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

Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition affecting fewer than 200,000 people in the U.S. A company that first obtains FDA approval for a designated orphan drug for the specified rare disease or condition generally receives orphan drug marketing exclusivity for that drug for a period of seven years from the date of its approval. This orphan drug exclusivity prevents the approval of another drug containing the same active moiety used for the same orphan indication, except in circumstances where, based on the FDA's determination, a subsequent drug is safer, more effective or makes a major contribution to patient care, or if the orphan drug manufacturer is unable to assure that a

sufficient quantity of the orphan drug is available to meet the needs of patients with the rare disease or condition. Orphan drug exclusivity may also be lost if the FDA later determines that the initial request for designation was materially defective. EXONDYS 51 was granted orphan drug exclusivity in the U.S. through September 19, 2023 for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. However, such exclusivity may not effectively protect the product from competition if the FDA determines that a subsequent drug containing the same active moiety for the same indication is safer, more effective or makes a major contribution to patient care, or if we are unable to assure the FDA that sufficient quantities of EXONDYS 51 are available to meet patient demand. In addition, orphan drug exclusivity does not prevent the FDA from approving competing drugs for the same or similar indication containing a different active moiety or from approving a drug containing the same active moiety for a different indication. If a subsequent drug is approved for marketing for the same or similar indication, we may face increased competition, and our revenues from the sale of EXONDYS 51 will be adversely affected.

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

Physicians are permitted to prescribe drug products for uses that are not described in the product's labeling and that differ from those approved by applicable regulatory agencies. Off-label uses are common across medical specialties. Although the FDA and other regulatory agencies do not regulate a physician's choice of treatments, the FDA and other regulatory agencies do generally prohibit advertising and promotion of off-label uses of approved drug products or promotion of an approved drug on information that is not in the final, FDA-approved label for a product and restrict communications on off-label use. Accordingly, we may not promote EXONDYS 51 in the U.S. for use in any indications other than for the treatment of DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. Additionally, we face limitations on our ability to promote EXONDYS 51 based on any information that is not included in the final FDA-approved label, including previously published clinical data. The FDA and other regulatory authorities actively enforce laws and regulations prohibiting promotion of a product for off-label uses and the promotion of products for which marketing approval has not been obtained. A company that is found to have improperly promoted its drug product will be subject to significant liability, including civil and administrative remedies as well as criminal sanctions.

Notwithstanding the regulatory restrictions on off-label promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non-misleading and non-promotional scientific exchange concerning their products and recent draft FDA guidance suggests that there are circumstances in which the FDA would not object to the promotion of certain information that is not included in the approved labeling but that is consistent with the approved labeling. We intend to engage in medical education activities and communicate with healthcare providers in compliance with all applicable laws, regulatory guidance and industry best practices. Although we have established a compliance program and continue to enhance it to ensure that all such activities are performed in a legal and compliant manner, EXONDYS 51 is our first commercial product which could increase risk of non-compliance with our internal compliance policies and applicable rules and regulations, which could negatively impact our business.

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

Other than EXONDYS 51, which the FDA approved for use in the U.S. in September 2016 and for which we filed an MAA in November 2016 with the EMA, our most advanced product candidates are exon 45- and 53-skipping products (casimersen and golodirsen, respectively), PPMO DMD exon 51 skipping product (SRP-5051), and Nationwide Children's Hospital's micro-dystrophin gene therapy program and Galgt2 gene therapy program.

We are in the process of conducting, starting or planning various EXONDYS 51 clinical trials, including trials that are required to comply with regulatory NDA and/or MAA filing requirements as well as studies we need to conduct to comply with our post-marketing FDA requirements/commitments to verify and describe clinical benefit. The exon 53-skipping product candidate, which we are working on with the SKIP-NMD consortium, is currently in the clinic. The Part I dose-titration portion of this Phase 1/2a study has been completed and Part II open label portion of the study is ongoing. We have also completed the dose titration portion and are conducting the open-label portion of a study for our exon 45-skipping product candidate. Additionally, we are enrolling patients for a clinical trial using exon 45- and 53-skipping product candidates, which we refer to as the ESSENCE study. We have also initiated a first in human study for PPMO DMD exon 51 (SRP-5051).

Nationwide Children's Hospital, with whom we are collaborating, initiated Phase 1/2a clinical trials for their micro-dystrophin gene therapy program and their Galgt2 gene therapy program. On June 19, 2018, Nationwide Children's Hospital presented positive preliminary results from its Phase 1/2a micro-dystrophin gene therapy clinical trial in the first three individuals with DMD enrolled to the trial. In addition, Myonexus, with whom we entered into a warrant to purchase common stock of Myonexus, is expected to initiate a Phase 1/2a trial for its MYO-101 program in the third quarter of 2018. The MYO-101 program, as well as four other, less advanced, Myonexus programs, aim to develop gene therapy-based treatments for various forms of LGMD.

The remainder of our product candidates are in discovery or early stages of development. These product candidates will require significant further development, financial resources and personnel to develop into commercially viable products and obtain regulatory approval, if at all. Given the FDA approval of EXONDYS 51, we expect that much of our effort and many of our

expenditures over the next several years will be devoted to clinical development and regulatory activities associated with EXONDYS 51 and other exon-skipping candidates as part of our larger follow-on exon strategy in DMD, our other disease candidates, our proprietary chemistry, and other potential therapeutic areas that provide long-term market opportunities. We may be delayed, restricted, or unable to further develop our active and other product candidates or successfully obtain approvals needed to market them. Although EXONDYS 51 was approved under accelerated approval by the FDA in the U.S., we may not be able to obtain an approval of EXONDYS 51 in the EU.

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

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

Our pre-clinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, including those based on our PMO-based and gene therapy-based technologies, which could prevent or significantly delay their regulatory approval.

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive pre-clinical and clinical 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 the pre-clinical data for PPMO collected to date is promising, the additional data we collect, including in the clinic, may not be consistent with the pre-clinical data or show a safe benefit that warrants further development or pursuit of a regulatory approval for PPMO product candidates. Furthermore, success in pre-clinical and early clinical trials does not ensure that the subsequent trials will be successful, nor does it predict final results of a confirmatory trial. For example, on June 19, 2018, Nationwide Children's Hospital presented positive preliminary results from its Phase 1/2a micro-dystrophin gene therapy clinical trial in the first three individuals with DMD enrolled to the trial. The preliminary data is based on a small patient sample and reported before completion of the trial and therefore may not be predictive of future results. In addition, we cannot assure that the results of additional preliminary data or data from the completed trial or any future trial will yield results that are consistent with the preliminary data presented, that we will be able to demonstrate the safety and efficacy of AAVrh74.MHCK7.micro-Dystrophin, 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 AAVrh74.MHCK7.micro-Dystrophin. Similarly, we cannot provide assurances that data from our studies with respect to EXONDYS 51, golodirsen, casimersen and gene therapy-based product candidates will be positive and consistent through the study periods or that the interpretation by regulators, such as the FDA or EMA, of the data we collect for our product or product candidates will be consistent with our interpretations.

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 regulatory approval.

If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, including for those that are based on our PMO-based technologies, then the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld or withdrawn. The completion of pre-clinical and clinical trials and regulatory approvals may be delayed for other reasons, such as delays related to patients enrollment for reasons including small patient population, competing clinical trials and patients' concerns regarding trial design; manufacturing of product candidates; and clinical holds, such as the Clinical Hold described above.

If there are significant delays in obtaining or we are unable to obtain or maintain required regulatory approvals, we will not be able to commercialize our product candidates in a timely manner or at all, which could impair our ability to generate sufficient revenue and have a successful business.

The research, testing, manufacturing, labeling, approval, commercialization, marketing, selling and distribution of drug products are subject to extensive regulation by applicable local, regional and national regulatory authorities and regulations may differ from jurisdiction to jurisdiction. In the U.S., approvals and oversight from federal (e.g., FDA), state and other regulatory authorities are required for these activities. Sale and marketing of our product candidates in the U.S. or other countries is not permitted until we obtain the required approvals from the applicable regulatory authorities. Our ability to obtain the government or regulatory approvals required to commercialize any of our product candidates in any jurisdiction, including in the U.S. or the EU, cannot be assured, may be significantly delayed or may never be achieved for various reasons including the following:

- Our non-clinical, clinical, chemistry, manufacturing and controls and other data and analyses from past, current and future studies for any of our product candidates may not be sufficient to meet regulatory requirements for marketing application approvals. The regulatory authorities could disagree with our interpretations and conclusions regarding data we provide in connection with NDA or MAA submissions for one or more of our product candidates, and may delay, reject or refuse to accept for review, or approve any NDA or MAA submission we make or identify additional requirements for product approval to be submitted upon completion, if ever. In addition, in the U.S., an FDA advisory committee could determine that our data are insufficient to provide a positive recommendation for approval of any NDA we submit to the FDA. Even if we meet FDA requirements and an advisory committee votes to recommend approval of an NDA submission, the FDA could still disagree with the advisory committee's recommendation and deny approval of a product candidate based on their review.
- The regulatory approval process for product candidates targeting orphan diseases, such as DMD, that use new technologies and processes, such as antisense oligonucleotide therapies, and alternative approaches or endpoints for the determination of efficacy is uncertain due to, among other factors, evolving interpretations of a new therapeutic class, the broad discretion of regulatory authorities, lack of precedent, 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. With respect to our gene therapy-based product candidates, although the FDA has encouraged sponsors to design first-in-patient studies as potential pivotal trials in a recent draft FDA guidance on gene therapy in rare disease, there is no assurance that we will be able to rely on this guidance to expedite the development of our gene therapy-based product candidate, including Nationwide Children's Hospital's Phase 1/2a micro-dystrophin gene therapy clinical trial. Limited data exist regarding the safety and efficacy of gene therapy-based therapeutics, and government regulation of such therapeutics is still evolving. We cannot be sure that any of our product candidates will qualify for accelerated approval or any other expedited development, review and approval programs, or that, if a drug does qualify, that the product candidates will be approved, will be accepted as part of any such program or that the review time will be shorter than a standard review. As a result of uncertainty in the approval process for products intended to treat serious rare diseases, we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned NDAs and MAAs for our product candidates, in a timely manner, or at all. Examples of such requests or requirements could include, but are not limited to, conducting additional or redesigned trials and procedures (e.g., additional safety data, patient muscle biopsies and dystrophin analyses), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, in the U.S., an FDA advisory committee or regulators may disagree with our data analysis, interpretations and conclusions at any point in the approval process, which could negatively impact the approval of our NDA or result in a decision by the Company not to proceed with an NDA submission for a product candidate based on feedback from regulators.
- We may not have the resources required to meet regulatory requirements and successfully navigate what is generally a lengthy, expensive and extensive approval process for commercialization of drug product candidates. Any failure on our part to respond to these requirements in a timely and satisfactory manner could significantly delay or negatively impact confirmatory study timelines and/or the development plans we have for golodirsen, casimersen, 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 or MAA submissions.

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

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

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

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

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

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

Responding to government investigations, defending any claims raised, and any resulting fines, restitution, damages and penalties, settlement payments or administrative actions, as well as any related actions brought by stockholders or other third parties, could have a material impact on our reputation, business and financial condition and divert the attention of our management from operating our business.

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

In connection with the commercial launch of EXONDYS 51, we are in the process of expanding our compliance program, which is based on industry best practices and is designed to ensure that our commercialization of EXONDYS 51 complies with all applicable laws, regulations and industry standards. As the requirements in this area are constantly evolving, we cannot be certain that our program will eliminate all areas of potential exposure. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. Any action against us for violation of these laws, even if we successfully defend against such action, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security, fraud and reporting laws may prove costly.

The EU has enacted a new data privacy regulation, the General Data Protection Regulation, a violation of which could subject us to significant fines.

In May 2018, a new privacy regime, the General Data Protection Regulation ("GDPR") will take effect and immediately be binding across all member states of the European Economic Area ("EEA"). The GDPR increases our obligations with respect to clinical trials conducted in 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 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 and gene therapy-based 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.

We also have relied upon and plan to continue to rely upon third-party contract research organizations ("CROs") to monitor and manage data 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.

In addition, the use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that this information will be misappropriated. 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 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.

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

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

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

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

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

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

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

We incurred an operating loss of $103.4 million and $134.2 million for the three and six months ended June 30, 2018, respectively. Our accumulated deficit was $1.4 billion as of June 30, 2018. Although we launched EXONDYS 51 in the U.S. in September 2016, we believe that it will take us some time to attain profitability and positive cash flow from operations. We have generally incurred expenses related to research and development of our technologies and product candidates, from general and administrative expenses that we have incurred while building our business infrastructure. We anticipate that our expenses will increase substantially if and/or as we:

- continue our launch and commercialization of EXONDYS 51 in the U.S.;
- expand the global footprint of EXONDYS 51 outside of the U.S.;
- establish our sales, marketing and distribution capabilities;
- continue our research, pre-clinical and clinical development of our product candidates;
- respond to and satisfy requests and requirements from regulatory authorities in connection with development and potential approval of our product candidates;
- initiate additional clinical trials for our product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- acquire or in-license other product candidates;
- maintain, expand and protect our intellectual property portfolio;
- increase manufacturing capabilities including capital expenditures related to our real estate facilities and entering into manufacturing agreements;
- hire additional clinical, quality control and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

As a result, we expect to continue to incur significant operating losses at least through 2018. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when, or if, we will become profitable.

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

We will likely require additional capital from time to time in the future in order to meet FDA post-marketing approval requirements and market and sell EXONDYS 51 as well as continue the development of product candidates in our pipeline, to expand our product portfolio and to continue or enhance our business development efforts. The actual amount of funds that we may need and the sufficiency of the capital we have or are able to raise will be determined by many factors, some of which are in our control and others that are beyond our control. The Company and our board of directors continue to assess optimization in the size and structure of the Company as well as in its strategic plans. For example, in March 2016, we announced a long-term plan to consolidate facilities within Massachusetts and closing our Corvallis, Oregon offices by end of that year. In June 2017, we announced the opening of our research and manufacturing center in Andover, Massachusetts. In addition, we recently established our European headquarters in Zug, Switzerland. Any failure on our part to strategically and successfully manage the funds we raise, with respect to factors within our control, could impact our ability to successfully commercialize EXONDYS 51 and continue developing our product candidates. Some of the factors partially or entirely outside of our control that could impact our ability to raise funds, as well as the sufficiency of funds the Company has to execute its business plans successfully, include the success of our research and development efforts, the status of our pre-clinical and clinical testing, costs and timing relating to securing regulatory approvals and obtaining patent rights, regulatory changes, competitive and technological developments in the market, regulatory decisions, and any commercialization expenses related to any product sales, marketing, manufacturing and distribution. An unforeseen change in these factors, or others, might increase our need for additional capital.

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

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

Further, we may also enter into relationships with pharmaceutical or biotechnology companies to perform research and development with respect to our technologies, research programs, conduct clinical trials or market our product candidates. Other than pre-clinical collaborations with academic or research institutions and government entities for the development of additional exon-skipping product candidates for the treatment of DMD, we currently do not have a strategic relationship with a third party to perform research or development using our technologies or assist us in funding the continued development and commercialization of any of our programs or product candidates. If we were to have such a strategic relationship, such third party may require us to issue equity to such third party, relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates, or to grant licenses on terms that may not be favorable to us.

Our indebtedness resulting from our Amended and Restated Credit and Security Agreement and new Revolving Credit Agreement and security agreement with MidCap could adversely affect our financial condition or restrict our future operations.

On July 18, 2017, we entered into (i) an the Amended and Restated Credit and Security Agreement with MidCap that provides a term loan of $60.0 million, (ii) the Revolving Credit Agreement that provides a revolving loan commitment of $40.0 million (which may be increased by an additional tranche of $20.0 million), (iii) an amendment to the pledge agreement related to the Amended and Restated Credit and Security Agreement and (iv) a pledge agreement related to the Revolving Credit Agreement. Our agreements with MidCap create limitations on us, including:

- requiring us to maintain pledge cash and certain other assets in favor of MidCap during the term of the agreements;
- limiting our flexibility in planning for, or reacting to, changes in our business and our industry;
- placing us at a competitive disadvantage compared to our competitors who have less debt or competitors with comparable debt at more favorable interest rates;
- limiting our ability to borrow additional amounts for working capital, capital expenditures, research and development efforts, acquisitions, debt service requirements, execution of our business strategy and other purposes; and
- resulting in an acceleration of the maturity of such term loans upon the occurrence of a material adverse change or another default under the agreements with MidCap.

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

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

Our consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the U.S. The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. Such estimates and judgments include 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, which could, in turn, subject us to securities class action litigation. Defending against such potential litigation relating to a restatement of our consolidated financial statements would be expensive and would require significant attention and resources of our management. Moreover, our insurance to cover our obligations with respect to the ultimate resolution of any such litigation may be inadequate. As a result of these factors, any such potential litigation could have a material adverse effect on our financial results and cause our stock price to decline, which could in turn subject us to securities class action litigation.

Comprehensive tax reform in the United States could adversely affect our business and financial condition.

The Tax Cuts and Jobs Act (the "TJCA") was enacted on December 22, 2017 in the United States. The TJCA 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.

Notwithstanding the reduction in the corporate income tax rate, the overall impact of the TJCA is uncertain, and our business and financial condition could be adversely affected. We are still in the process of evaluating the TJCA and do not know the full effect it will have on our business, including our consolidated financial statements. The TJCA is complex and far-reaching and we cannot predict with certainty the impact its enactment will have on us. Moreover, that effect, whether adverse or favorable, may not become evident for some period of time. Further, we urge stockholders to consult with their legal and tax advisors with respect to the Tax Reform Act and the potential tax consequences of investing in our common stock.

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

In general, under Section 382 of the Internal Revenue Code, a corporation that undergoes an ''ownership change'' is subject to limitations on its ability to utilize its pre-change net operating losses and certain other tax assets to offset future taxable income. In general, an ownership change occurs if the aggregate stock ownership of certain stockholders increases by more than 50 percentage points over such stockholders' lowest percentage ownership during the testing period, which is generally three years. An ownership change could limit our ability to utilize our net operating loss and tax credit carryforwards for taxable years including or following such "ownership change." Limitations imposed on the ability to use net operating losses and tax credits to offset future taxable income could require us to pay U.S. federal income taxes earlier than we estimated or than would have otherwise been required if such limitations were not in effect and could cause such net operating losses and tax credits to expire unused, in each case reducing or eliminating the benefit of such net operating losses and tax credits and potentially adversely affecting our financial position. Similar rules and limitations may apply for state income tax purposes.

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

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

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

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

We anticipate continued growth in our business operations due, in part, to the commercialization of EXONDYS 51. This future growth could create a strain on our organizational, administrative and operational infrastructure. Our ability to manage our growth properly and maintain compliance with all applicable rules and regulations will require us to continue to improve our operational, legal, financial and management controls, as well as our reporting systems and procedures. We may not be able to build or maintain the management and human resources and infrastructure necessary to support the growth of our business. The time and resources required to implement systems and infrastructure that may be needed to support our growth is uncertain, and failure to complete implementation in a timely and efficient manner could adversely affect our operations.

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

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

Our success, competitive position and future revenue depend in part on our ability and the abilities of our licensors and other collaborators to obtain and maintain patent protection for our technologies, product and product candidates, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing on the proprietary rights of third parties.

We currently 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 platform technology, product and product candidates, including EXONDYS 51, golodirsen, casimersen, SRP-5051 as well as our gene therapy-based product candidates (micro-dystrophin and GALGT2). We anticipate filing additional patent applications both in the U.S. and in other countries. The patent process, however, is subject to numerous risks and uncertainties, and we can provide no assurance that we will be successful in obtaining and defending patents or in avoiding infringement of the rights of others. Even when our patent claims are allowed, the claims may not issue, or in the event of issuance, may not be sufficient to protect the technology owned by, optioned, or licensed to us or our collaborators. Even if our patents and patent applications do provide our product, product candidates and platform technology with a basis for exclusivity, we and our collaborators may not be able to develop or commercialize such product and product candidates (whether PMO-based or gene therapy-based) or platform technology due to patent positions held by one or more third parties.

We may not be able to obtain and maintain patent protection for our product or product candidates necessary to prevent competitors from commercializing competing product candidates. Our patent rights might be challenged, invalidated, circumvented or otherwise not provide any competitive advantage, and we might not be successful in challenging the patent rights of our competitors through litigation or administrative proceedings. Additionally, in order to maintain or obtain freedom to operate for our products and product candidates (whether PMO-based or gene therapy-based), we may incur significant expenses, including those associated with entering into agreements with third parties that require milestone and royalty payments. For example, in July 2017, we and The University of Western Australia on the one hand, and the BioMarin Parties and AZL on the other hand, executed a Settlement Agreement pursuant to which all existing efforts pursuing ongoing litigation, opposition and other administrative proceedings would be stopped as between the Settlement Parties and the Settlement Parties would cooperate to withdraw the Actions before the European Patent Office (except for actions involving third parties), the United States Patent and Trademark Office ("USPTO"), the U.S. Court of Appeals for the Federal Circuit and the High Court of Justice of England and Wales, except for the cross-appeal of the Interlocutory Decision of the Opposition Division dated April 15, 2013 of the European Patent Office of EP 1619249B1 in which we withdrew our appeal and the BioMarin Parties and AZL will continue with its appeal, with us having the right to provide input on the appeal. Any adverse rulings on the appeal, or any of the Actions that continue irrespective of the settlement, could come at any time and, if negative, could adversely affect our business and result in a decline in our stock price. Defending our patent positions may continue to require significant financial resources and could negatively impact other Company objectives. In addition, the expected benefits and opportunities related to the Settlement Agreement and the License Agreement may not be realized or may take longer to realize than expected due to challenges and uncertainties regarding the sales of EXONDYS 51, the research and development of future exon-skipping products, BioMarin's retained rights to convert the exclusive patent license under the Settlement Agreement to a co-exclusive license, BioMarin continuing certain oppositions and appeals, and patent oppositions that have been filed by other third parties, and patent oppositions and other patent challenges that may be filed by third parties in the future.

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 product and product candidates and gene therapy-based product candidates for which there has been little patent litigation 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. In addition, there is no assurance as to the degree and range of protections any of our patents, if issued, may afford us or whether patents will be issued. Patents which may be issued to us may be subjected to further governmental review that may ultimately result in the reduction of their scope of protection, and pending patent applications may have their requested breadth of protection significantly limited before being issued, if issued at all. The pharmaceutical, biotechnology and other life sciences patent situation outside the U.S. can be even more uncertain.

The DMD patent landscape is continually evolving, and we may be able to assert that certain activities engaged in by third parties infringe on our current or future patent rights. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights. 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. In the U.S., our patents may be challenged in an Inter Partes Review proceeding or other related proceeding. In other countries, other procedures are available for a third party to challenge the validity of our patent rights. For instance, we have rights to European Patent No. 2206781, which protects golodirsen. This patent was opposed at the European Patent Office. On December 19, 2017, the Opposition Division issued a Decision ordering the revocation of this patent. We have appealed this Decision. Patents we have rights to from BioMarin that cover our PMO-based candidates including golodirsen are involved in third party opposition proceedings in Europe and Japan. These patents that we are defending in third party opposition proceedings, however, are not expected to be the sole basis for exclusivity for our product candidates, if at all, in view of their standard expiration dates.

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. For instance, a group that includes Knowledge Ecology International ("KEI") sent a letter to the U.S. Department of Health and Human Services ("HHS") requesting that HHS take title to five patents that cover eteplirsen under the Bayh-Dole Act as a remedy for allegedly failing to disclose NIH funding of inventions resulting from NIH grants. An investigation into the allegations by KEI is ongoing. Additionally, jurisdictions other than the U.S. might have less restrictive patent laws than the U.S., giving foreign competitors the ability to exploit these laws to create, develop and market competing products. The USPTO and patent offices in other jurisdictions have often required that patent applications concerning pharmaceutical and/or biotechnology-related inventions be limited or narrowed substantially to cover only the specific innovations exemplified in the patent application, thereby limiting the scope of protection against competitive challenges. Accordingly, even if we or our licensors are able to obtain patents, the patents might be substantially narrower than anticipated.

On September 16, 2011, the Leahy-Smith America Invents Act (the "Leahy-Smith Act"), was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted, and may also affect patent litigation. The USPTO has issued regulations and procedures to govern administration of the Leahy-Smith Act, but many of the substantive changes to patent law associated with the Leahy-Smith Act have only recently become effective. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition. For instance, a third party may petition the PTAB seeking to challenge the validity of some or all of the claims in any of our patents through an *Inter Partes Review* ("IPR") or other post-grant proceeding. Should the PTAB institute an IPR (or other) proceeding and decide that some or all of the claims in the challenged patent are invalid, such a decision, if upheld on appeal, could have a material adverse effect on our business and financial condition.

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

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

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

If EXONDYS 51 or our product candidates (whether PMO-based or gene therapy-based) or technologies infringe enforceable proprietary rights of others, we could incur substantial costs and may have to:

- obtain rights or licenses from others, which might not be available on commercially reasonable terms or at all;
- abandon development of an infringing product candidate;
- redesign EXONDYS 51, product candidates or processes to avoid infringement;

- pay damages; and/or
- defend litigation or administrative proceedings which might be costly whether we win or lose, and which could result in a substantial diversion of financial and management resources.

Any of these events could substantially harm our potential earnings, financial condition and operations. The DMD patent landscape (whether PMO-based or gene therapy-based) is continually evolving and multiple parties, including both commercial entities and academic institutions, may have rights to claims or may be pursuing additional claims that could provide these parties a basis to assert that EXONDYS 51 or our product candidates infringe on the intellectual property rights of such parties. Similarly, we may be able to assert that certain activities engaged in by these parties infringe on our current or future patent rights. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights. We also cannot be certain that other third parties will not assert patent infringement in the future with respect to any of our development programs.

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

The biotechnology and pharmaceutical industries are highly competitive and subject to significant and rapid technological change. We are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development in areas related to antisense technology and other technologies, or that are developing alternative approaches to or therapeutics for the disease indications on which we are focused. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with EXONDYS 51 or our product candidates. For example, we believe that companies including Alnylam Pharmaceuticals, Inc., Ionis Pharmaceuticals, Inc. (formerly Isis Pharmaceuticals, Inc.), Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Wave Life Sciences, Daiichi Sankyo and Nippon share a focus on RNA-targeted drug discovery and development. Competitors with respect to EXONDYS 51 or our product candidates (whether PMO-based or gene therapy-based) include Nippon Shinyaku, Daiichi Sankyo, Wave Life Sciences, Solid, Pfizer, Shire plc; and other companies such as PTC have also been working on DMD programs. Additionally, several companies and institutions have entered into collaborations or other agreements for the development of product candidates, including mRNA, gene (CRIPSR and AAV, among others) and small molecule therapies that are potential competitors for therapies being developed in the muscular dystrophy, neuromuscular and rare disease space, including, but not limited to, Biogen Inc., Ionis, Alexion Pharmaceuticals, Inc., Sanofi, Shire, Eli Lilly, Alnylam Pharmaceuticals, Inc., Moderna Therapeutics, Inc., Summit, Akashi, Catabasis, Capricor Therapeutics, Oxford University, Exonics Therapeutics, and Editas Medicine. Although BioMarin announced on May 31, 2016 its intent to discontinue clinical and regulatory development of drisapersen as well as its other clinical stage candidates, BMN 044, BMN 045 and BMN 053, then-currently in Phase 2 studies for distinct forms of DMD, it further announced its intent to continue to explore the development of next generation oligonucleotides for the treatment of DMD.

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

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

- develop safer or more effective products;
- implement more effective approaches to sales and marketing;
- develop less costly products;
- obtain regulatory approval more quickly;
- have access to more manufacturing capacity;
- develop products that are more convenient and easier to administer;
- form more advantageous strategic alliances; or
- establish superior intellectual property positions.

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

The current and future use of our product candidates by us and our collaborators in clinical trials, expanded access programs, the sale of EXONDYS 51 and future products, or the use of our products under emergency use vehicles may expose us to liability claims inherent to the manufacture, clinical testing, marketing and sale of medical products. These claims might be made directly by consumers or healthcare providers or indirectly by pharmaceutical companies, our collaborators or others selling such products. Regardless of merit or eventual outcome, we may experience financial losses in the future due to such product liability claims. We have obtained limited general commercial liability insurance coverage for our clinical trials. We intend to expand our insurance coverage to include the sale of commercial products in connection with the FDA's approval of EXONDYS 51. However, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against all losses. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

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

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

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

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, our proprietary business information and that of our suppliers, as well as personally identifiable information of EXONDYS 51 patients, clinical trial participants and employees. Similarly, our third party providers possess certain of our sensitive data. The secure maintenance of this information is critical to our operations and business strategy. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. Any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information, including our data being breached at third party providers, could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations and damage our reputation, which could adversely affect our business.

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

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

Risks Related to Our Common Stock

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

The market prices for and trading volumes of securities of biotechnology companies, including our securities, has historically been volatile. Our stock has had significant swings in trading prices, in particular in connection with our public communications regarding feedback received from regulatory authorities. For example, over the last thirty months, our stock has increased as much as 74% in a single day or decreased as much as 55% in a single day. The market has from time to time experienced significant price and volume fluctuations unrelated to the operating performance of particular companies. The market price of our common stock may fluctuate significantly due to a variety of factors, including but not limited to:

- the commercial performance of EXONDYS 51 in the U.S.;
- the timing of our submissions to regulatory authorities and regulatory decisions and developments;
- positive or negative clinical trial results or regulatory interpretations of data collected in clinical trials conducted by us, our strategic partners, our competitors or other companies with investigational drugs targeting the same, similar or related diseases to those targeted by us;
- delays in beginning and completing pre-clinical and clinical trials for potential product candidates;
- delays in entering or failing to enter into strategic relationships with respect to development and/or commercialization of EXONDYS 51 or our product candidates or entry into strategic relationships on terms that are not deemed to be favorable to our Company;
- technological innovations, product development or additional commercial product introductions by ourselves or competitors;
- changes in applicable government regulations or regulatory requirements in the approval process;
- developments concerning proprietary rights, including patents and patent litigation matters, such as developments in the interferences declared by the USPTO, including in the near term any outcomes of ongoing interference proceedings and over the longer term the outcomes from any related appeals;
- public concern relating to the commercial value, efficacy or safety of any of our products;
- our ability to obtain funds, through the issuance of equity or equity linked securities or incurrence of debt, or other corporate transactions;
- comments by securities analysts;
- developments in litigation such as the stockholder lawsuits against us;
- changes in senior management; or
- general market conditions in our industry or in the economy as a whole.

Broad market and industry factors may seriously affect the market price of a company's stock, including ours, regardless of actual operating performance. In addition, in the past, following periods of volatility in the overall market and the market price of a particular company's securities, securities class action litigation has often been instituted against these companies. Such litigation could result in substantial costs and a diversion of our management's attention and resources.

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

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

- when the board is comprised of six or more directors, classification of our board of directors into two classes, with one class elected each year;
- directors may only be removed for cause by the affirmative vote of a majority of the voting power of all the then-outstanding shares of voting stock;
- prohibition of cumulative voting of shares in the election of directors;

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

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

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 my 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 Centers for Medicare & Medicaid Services (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;
- introduction of new products by others that render our product obsolete or noncompetitive;
- the ability to maintain selling prices and gross margin on our product;
- increases in the cost of raw materials contained within our product;
- 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; 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.

A significant number of shares of our common stock are issuable pursuant to outstanding stock awards, and we expect to issue additional stock awards and shares of common stock in the future. Exercise of these awards and sales of shares will dilute the interests of existing security holders and may depress the price of our common stock.

As of June 30, 2018, there were approximately 66.3 million shares of common stock outstanding and outstanding awards to purchase 9.4 million shares of common stock under various incentive stock plans. Additionally, as of June 30, 2018, there were approximately 4.2 million shares of common stock available for future issuance under our 2018 Equity Incentive Plan, approximately 0.2 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan, and approximately 0.1 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan. We may issue additional common stock and warrants from time to time to finance our operations. We may also issue additional shares to fund potential acquisitions or in connection with additional stock options or other equity awards granted to our employees, officers, directors and consultants under our 2018 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. The issuance of additional shares of common stock or warrants to purchase common stock and the perception that such issuances may occur or exercise of outstanding warrants or options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

Risks Related to Our Convertible Senior Notes

Servicing our 1.50% notes due 2024 (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 million aggregate principal amount of Notes. 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 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 our Notes, which are non-callable and mature in 2024, will depend on the capital markets and our financial condition at such time. 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 our 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 entered into various derivative transactions with respect to our common stock and/or to purchase 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 could also cause or avoid an increase or a decrease in the market price of our common stock or the Notes, which could affect the value of our common stock.

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.

None.

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	
2.1*	Warrant to Purchase Common Stock of Myonexus Therapeutics, Inc., issued by Myonexus Therapeutics, Inc. to Sarepta Therapeutics, Inc., dated as of May 3, 2018.					X
10.1†	Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan					X
10.2†	Employment Agreement between Sarepta Therapeutics, Inc. and Gilmore O'Neill, M.D., effective as of June 7, 2018					X
10.3†	Change in Control and Severance Agreement between Sarepta Therapeutics, Inc. and Gilmore O'Neill, M.D., effective as of June 7, 2018					X
10.4†	Letter Agreement between Douglas S. Ingram and Sarepta Therapeutics, Inc. dated June 26, 2018					X
10.5†	Form of Restricted Stock Unit Award Agreement under Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan					X
10.6†	Amendment No. 2 to the Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan					X
31.1	Certification of the Company's Chief Executive Officer, Douglas S. Ingram, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company's Executive Vice President, Chief Financial Officer and Chief Business Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1**	Certification of the Company's Chief Executive Officer, Douglas S. Ingram, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2**	Certification of the Company's Executive Vice President, Chief Financial Officer and Chief Business Officer, Sandesh Mahatme, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	XBRL Instance Document.					X
101.SCH	XBRL Taxonomy Extension Schema Document.					X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.					X

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

* Confidential treatment has been requested for portions of this exhibit. Schedules and exhibits of this exhibit have been omitted pursuant to Item 601(b)(2) of Regulation S-K. The Company agrees to furnish a copy of an omitted schedule or exhibit to the Securities and Exchange Commission upon request.

** 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 8, 2018

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

Date: August 8, 2018

By: /s/ SANDESH MAHATME
Sandesh Mahatme
Executive Vice President,
Chief Financial Officer and
Chief Business Officer
(Principal Financial and Accounting Officer)

EXHIBIT 2.1

[****] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Securities and Exchange Commission.

EXECUTION VERSION

WARRANT TO PURCHASE COMMON STOCK
OF

MYONEXUS THERAPEUTICS, INC.

[****] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Securities and Exchange Commission.

TABLE OF CONTENTS

[****] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Securities and Exchange Commission.

Exhibit List

[****] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Securities and Exchange Commission.

THIS WARRANT AND THE SECURITIES PURCHASABLE HEREUNDER HAVE NOT BEEN REGISTERED OR QUALIFIED UNDER THE SECURITIES ACT OF 1933, AS AMENDED, OR UNDER ANY APPLICABLE STATE SECURITIES LAWS, AND HAVE BEEN ACQUIRED FOR INVESTMENT AND NOT WITH A VIEW TO, OR IN CONNECTION WITH, THE SALE OR DISTRIBUTION THEREOF. NO SUCH SALE OR DISTRIBUTION MAY BE LAWFULLY EFFECTED WITHOUT AN EFFECTIVE REGISTRATION STATEMENT OR QUALIFICATION RELATED THERETO OR AN OPINION OF COUNSEL IN A FORM SATISFACTORY TO THE COMPANY THAT SUCH REGISTRATION IS NOT REQUIRED.

MYONEXUS THERAPEUTICS, INC.

WARRANT TO PURCHASE COMMON STOCK

dated as of May 3, 2018

THIS CERTIFIES THAT, for valuable consideration as set forth in Section 2.1 and in accordance with the terms set forth in this Warrant, Sarepta Therapeutics, Inc. or its successors or assigns (such Person and such successors and assigns each being the "Warrant Holder" with respect to the Warrant held by it) is entitled (a) to purchase from Myonexus Therapeutics, Inc., a Delaware corporation (the "Company"), the number of Shares equal to the Purchase Amount for an aggregate purchase price equal to the Warrant Exercise Payment (as herein defined), and (b) to the other rights set forth herein.

IN FURTHERANCE THEREOF, and in consideration of the mutual covenants and agreements set forth herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Company and the Warrant Holder hereby agree as follows:

ARTICLE 1
DEFINITIONS AND CONSTRUCTION

Section 1.1. Definitions.

(a) As used herein (the following definitions being applicable in both singular and plural forms):

"280G Rules" is defined in Section 5.5(f).

"280G Stockholder Vote" is defined in Section 5.5(f).

"Affiliate" means, with respect to a Person, another Person that directly, or indirectly through one or more intermediaries, controls, or is controlled by, or is under common control with, such Person; provided that for purposes of this definition, "control" means, with respect to a Person, the possession, directly or indirectly, of the power to direct or cause the direction of the management and policies of such Person, whether through the ownership of voting securities, by Contract, or otherwise.

"Alternative Notice" is defined in Section 2.8(a).

"Alternative Terms" is defined in Section 2.8(a).

"Antitrust Approval" is defined in Section 5.18(b).

"Auditor" is defined in Section 2.7(b).

"Business Day" means any day except a Saturday, Sunday or other day on which banking institutions located in New York City are permitted or required by Law, executive order or decree of a Governmental Entity to remain closed.

"Capital Stock" means any capital stock or share capital of, other voting securities of, other equity interest in, or right to receive profits, losses or distributions of, any Person.

"Cash on Hand" means, with respect to a Person as of a particular time of determination, the aggregate cash of such Person at such time, determined in accordance with GAAP.

"CERCLA" means the Federal Comprehensive Environmental Response, Compensation, and Liability Act of 1980 (42 U.S.C. §§ 9601 et seq.), as amended, and the rules and regulations promulgated thereunder, and any foreign and state Law counterparts.

"Certificate of Incorporation" means the Company's Amended and Restated Certificate of Incorporation, as further amended and restated from time to time.

"Change of Control" means any direct or indirect acquisition or sale of substantially all of the assets of the Warrant Holder or direct or indirect acquisition of a majority of the voting stock of the Warrant Holder (whether through a share purchase, merger, consolidation, business combination, recapitalization or similar transaction involving the Warrant Holder) by a single Person or group (as defined in the U.S. federal securities laws).

"Change of Control Payments" means any amounts which become payable in cash or property by the Company to any of its current or former employees or consultants prior to, on, or following the Warrant Exercise Closing as a result of the execution and delivery of this Warrant, the purchase of Shares purchasable hereunder or consummation of any other transactions contemplated hereby (in each case, other than any payments made to any Person in accordance with the Redemption Provisions), whether pursuant to any Plan or severance policy of the Company or any individual employment, severance or change-of-control Contract or otherwise, and have not been paid prior to the Warrant Exercise Closing Date, plus **[****]**.

"Class G Preferred Stock" means the Company's Class G Preferred Stock, $0.0001 par value per share.

"Clinical Trial" means a research study in humans that is (i) conducted in accordance with international ethical and scientific quality standards for designing, conducting, recording and reporting research studies that involve the participation of human subjects, which standards are established through FDA guidance (including ICH E6) in the United States, and (ii) designed to generate data regarding a chemical compound or biological molecule in support or maintenance of an IND or NDA.

"Closing Balance Sheet" is defined in Section 2.7(b).

"Closing Date Cash and Liabilities Amount" means (i) the Cash on Hand of the Company as of 11:59 p.m. Eastern Standard Time on the day immediately prior to the date of the Warrant Exercise Closing (so long as such amount is a positive number) *plus* (ii) **[****]**, *less* (iii) any liabilities of the Company (including Pre-Closing Tax Liabilities (including an estimate of Pre-Closing Tax Liabilities for the portion of any Straddle Period ending on the date of the Warrant Exercise Closing)) included on the Closing Balance Sheet of the Company (determined, in each case, in accordance with GAAP, consistently applied in accordance with past practices) as of 11:59 p.m. Eastern Standard Time on the day immediately prior to the date of the Warrant Exercise Closing (except that Pre-Closing Tax Liabilities shall be determined as of the end of the day on the Warrant Exercise Closing Date) (the amount in clause (ii) to be expressed as a positive number).

"Closing Payment Decrease" is defined in Section 2.7(c).

"Closing Payment Increase" is defined in Section 2.7(c).

"Code" means the Internal Revenue Code of 1986, as amended, including any substitute or successor provisions.

"Collaboration Parties" is defined in Section 5.3(b)(xiii)(A).

"Commercially Reasonable Efforts" means, with respect to the efforts to be expended by a Party hereto, with respect to any task or objective under this Warrant, reasonable, diligent, good-faith efforts to accomplish such task or objective, which efforts shall not be less than the efforts that other similarly situated companies would normally use to accomplish a similar task or objective under similar circumstances exercising reasonable business judgment, and with respect to development or commercialization obligations, for a product owned by it or to which it has rights, that is of similar overall market potential and that is at a similar stage in its product lifecycle, taking into account, inter alia, the competitiveness of the marketplace, the proprietary position of the product, the regulatory structure involved, the profitability of the product and other relevant factors.

"Commission" means the Securities and Exchange Commission or any other Federal agency administering the Securities Act at the time.

"Company" is defined in the Preamble.

"Company Capital Stock" means the Capital Stock of the Company.

"Company Common Stock" means the Company's common stock, $0.0001 par value per share.

"Company Equityholder Indemnified Party" is defined in Section 7.2.

"Company Equityholders" means, collectively, the Company Stockholders and the holders of Company Warrants and holders of Company Stock Options, in each case, immediately prior to the Redemption Time (as such term is defined in the Certificate of Incorporation).

"Company Intellectual Property" means, collectively, all Owned Intellectual Property and Licensed Intellectual Property.

"Company Intellectual Property Contract" is defined in Section 3.14(e).

"Company's knowledge," "knowledge of the Company" or variations thereof means the actual knowledge of each of the **[****]**, in each case, after reasonable inquiry of other Company Personnel reasonably likely to have knowledge of the matter in question and reasonable inquiry of the Company's outside intellectual property counsel **[****]**.

"Company Patent" means any Patent owned or exclusively licensed to the Company at the time of the Warrant Exercise Closing.

"Company Personnel" means any director, officer, employee, independent contractor or consultant of the Company.

"Company Phase 1/2A Clinical Trial" means a Clinical Trial of a Product Candidate, conducted in accordance with the criteria set forth in the Development Plan and Budget set forth in Exhibit A.

"Company Preferred Stock" means each class or series of the Company's preferred stock, $0.0001 par value per share.

"Company Stock Option" means an option to purchase or acquire shares of Company Capital Stock issued pursuant to a Company Stock Plan.

"Company Stock Plan" means any stock option plan or other stock or equity-related plan of the Company, including, but not limited to, the Company's 2017 Equity Incentive Plan, as amended.

"Company Stockholder" means a holder of Company Capital Stock immediately prior to the Redemption Time (as such term is defined in the Certificate of Incorporation), other than the Warrant Holder.

"Company Warrant" means a warrant, option or other right to purchase or acquire Company Capital Stock, provided that Company Stock Options and this Warrant shall not be considered Company Warrants.

"Conclusion of the Escalation Process" is defined in Section 9.4(d).

"Confidential Information" is defined in Section 5.12.

"Confidentiality Agreement" means the Confidentialy [sic] Agreement effective May 1, 2017 by and between NCH, the Company and the Warrant Holder, as amended.

"Constitutive Documents" means the articles or certificate of incorporation and by-laws of a Person if such Person is a corporation, and analogous constitutive documents if such Person is another form of entity.

"Contingent Payment" is defined in Section 2.6(a).

"Contingent Payment Event" is defined in Section 2.6(a).

"Contract" means any loan or credit agreement, bond, debenture, note, mortgage, indenture, guarantee, security agreement, lease or other contract, agreement, instrument, license, evidence of Indebtedness or legally binding arrangement or understanding, whether written or oral.

"Convertible Noteholder" means each of PENSCO Trust Company, LLC, Custodian FBO Bryan Barber, Scott Frewing, Gruppo Familiari Beta-Sarcoglicanopatie Onlus, Rev1 NCH Fund I, LLC, Jain Foundation Inc., Bradley Williams, DYSF Investments LLC and CincyTech Fund IV, LLC, and, collectively, the "Convertible Noteholders".

"Covered Person" is defined in Section 5.21(a).

"Data Trigger Notification" is defined in Section 5.1.

"Deal Fees" means all fees and expenses (including fees and expenses of investment bankers, finders, consultants, attorneys, accountants or others) of the Company incurred or owed or reimbursable by the Company in connection with the negotiation and entering into of this Warrant, the issuance of Shares hereunder and the transactions contemplated hereunder (excluding the Development Program), in each case to the extent unpaid prior to or at the Warrant Exercise Closing.

"Development Milestone Event" means each of Development Milestone Event 1, Development Milestone Event 2, Development Milestone Event 3, Development Milestone Event 4 and Development Milestone Event 5.

"Development Milestone Event 1" means **[****]**.

"Development Milestone Event 2" means **[****]**.

"Development Milestone Event 3" means **[****]**.

"Development Milestone Event 4" means **[****]**. For this purpose "clearance by the FDA" means either (a) 30 days have passed since the filing with no clinical hold imposed, or issues raised, by the FDA so that the clinical trial can begin dosing, or (b) if the FDA has raised any issues, the issues have been resolved to the FDA's satisfaction so that the clinical trial can begin dosing.

"Development Milestone Event 5" means **[****]**. For this purpose "clearance by the FDA" has the meaning specified in the definition of Development Milestone Event 4.

"Development Milestone Payment" means each of Development Milestone Payment 1, Development Milestone Payment 2, Development Milestone Payment 3, Development Milestone Payment 4 and Development Milestone Payment 5.

"Development Milestone Event Notice" is defined in Section 2.1(b)(i).

"Development Milestone Payment 1" means an amount in cash equal to $**[****]**.

"Development Milestone Payment 2" means an amount in cash equal to $**[****]**.

"Development Milestone Payment 3" means an amount in cash equal to $**[****]**.

"Development Milestone Payment 4" means an amount in cash equal to $**[****]**.

"Development Milestone Payment 5" means an amount in cash equal to $**[****]**.

"Development Milestone Review Period" means, with respect to each Development Milestone Event, the period commencing on the date on which (a) the applicable Development Milestone Event Notice required by Section 2.1(b)(i) and (b) the certificate required by Section 2.1(c)(v) are given, and ending **[****]** Business Days later.

"Development Plan and Budget" or "Development Plan" means the budget and plan for the Development Program and research and development activities to be conducted by the Company during the Warrant Period, attached hereto as Exhibit A.

"Development Program" means the Company's pre-clinical and clinical development activities for the Product Candidates set forth in the Development Plan.

"Disclosure Schedule" means a schedule of exceptions to the representations and warranties of the Company set forth in Article 3, delivered either contemporaneously with this Warrant or pursuant to Section 2.2(a). The Disclosure Schedule shall be arranged in sections and subsections corresponding to the numbered and lettered sections and subsections contained in Article 3. The disclosures in any section or subsection of the Disclosure Schedule shall be deemed disclosure with respect to any other section or subsection of the Disclosure Schedule to which the relevance of such item is reasonably apparent on the face of that disclosure.

"Dispute" is defined in Section 9.4(a).

"Dispute Escalation Process" is defined in Section 9.4(a).

"DOJ" means the United States Department of Justice.

"EMA" means the European Medicines Agency or any successor agency or authority thereto.

"Environmental Law" means any Law relating to (i) the manufacture, processing, use, labeling, distribution, treatment, storage, discharge, disposal, recycling, generation or transportation of Hazardous Materials; (ii) air (including indoor air), soil, surface, subsurface, groundwater or noise pollution; (iii) Releases or threatened Releases; (iv) protection of wildlife, endangered species, wetlands or natural resources; (v) underground storage tanks (USTs); (vi) above-ground storage tanks (ASTs); (vii) health and safety of employees and other persons; (viii) the presence or content of Hazardous Materials in a product, item or article, whether a component or finished product; (ix) land use and zoning requirements; and (x) notification requirements relating to the foregoing. Without limiting the above, Environmental Law also includes the following within the United States and all foreign equivalents thereof: (A) CERCLA; (B) the Solid Waste Disposal Act, as amended by RCRA; (C) the Emergency Planning and Community Right to Know Act of 1986 (42 U.S.C. §§ I 101 et seq.), as amended; (D) the Clean Air Act (42 U.S.C. §§ 7401 et seq.), as amended; (E) the Clean Water Act (33 U.S.C. §§ 1251 et seq.), as amended; (F) the Toxic Substances Control Act (15 U.S.C. §§ 2601 et seq.), as amended; (G) the Hazardous Materials Transportation Act (49 U.S.C. §§ 1801 et seq.), as amended; (H) the Federal Insecticide, Fungicide and Rodenticide Act (7 U.S.C. §§ 136 et seq.), as amended; (I) the Federal Safe Drinking Water Act (42 U.S.C. §§ 300 et seq.), as amended; (J) the Federal Radon and Indoor Air Quality Research Act (42 U.S.C. §§ 7401 note, et seq.), as amended; (K) the Occupational Safety and Health Act (29 U.S.C. §§ 651 et seq.), as amended; and (L) any Laws similar or analogous to (including counterparts of) any of the statutes listed above in effect as of the date of the Warrant Exercise Closing.

"ERISA" means the Employee Retirement Income Security Act of 1974.

"ERISA Affiliate" is defined in Section 3.19(a).

"Estimated Closing Date Cash and Liabilities Amount" is defined in Section 2.7(a).

"Exchange Act" means the Securities Exchange Act of 1934, or any successor federal statute, and the rules and regulations of the Commission thereunder, as amended from time to time.

"Executive Officer" is defined in Section 6.2(b).

"Exercise Notice" is defined in Section 2.3(a).

"Exploratory IND or Exploratory CTA Study" means a single exploratory first in human clinical study that (i) is completed primarily to obtain pharmacokinetic or pharmacodynamic information as is described in "Guidance for Industry, Investigators and Reviewers - Exploratory IND Studies," dated January 2006, published by the FDA's Center for Drug Evaluation and Research, and (ii) is not associated with a clinical development plan, and (iii) the sponsor of which specifically states in the IND that the IND is intended to be withdrawn after completion of the outlined study and the sponsor withdraws the IND following such study, and (iv) involves a limited number of subjects with a limited range of doses for a limited period of time, where such doses are intended to be sub-pharmacologic and for which there is no reasonable expectation that the dose could produce a toxic effect. In the event that FDA withdraws the guidance without replacing it with provisions that are in all material respects identical to the guidance, no Clinical Trial thereafter shall qualify as an "Exploratory IND or Exploratory CTA Study" hereunder.

"FCPA" is defined in Section 3.26.

"FDA" means the U.S. Food and Drug Administration, or any successor agency or authority thereto.

"FDCA" means the United States Federal Food, Drug, and Cosmetic Act, as amended.

"Financial Statements" is defined in Section 3.8.

"Founders" means each of Bryan Barber, Bruce M. Halpryn, Louise Rodino-Klapac and Michael D. Triplett II.

"Fraud" means that a court of competent jurisdiction has concluded, in a final and non-appealable order, decree, ruling or other action, that a Party hereto has committed an act in connection with the representations and warranties contained in Article 3 of this Warrant with specific intent to induce such other party to enter into this Warrant or to give the Exercise Notice and requires (i) a false representation of material fact made herein; (ii) with knowledge that such representation is false when made; (iii) with the specific intention to induce the Party to whom such representation is made to act or refrain from acting in reliance upon it; (iv) causing that party, in justifiable reliance upon such false representation and with ignorance to the falsity of such representation, to take or refrain from taking action; and (v) causing such party to suffer damage by reason of such reliance. "Fraud" shall only include common law liability for fraud and shall expressly exclude legal theories such as equitable fraud, promissory fraud, unfair dealings fraud and other fraud-based claims.

"FTC" means the United States Federal Trade Commission.

"FTE" means a full-time equivalent person year (consisting of **[****]** hours per year) of scientific, technical, or commercialization work undertaken by the Company or the Warrant Holder employees, as applicable.

"FTE Costs" means, for any period, the FTE Rate multiplied by the number of FTEs in such period. FTEs will be pro-rated on a daily basis if necessary.

"FTE Rate" means the cost of the applicable FTE including salary, benefits, administration, facilities costs, and overhead (which may be prorated on a daily basis as necessary), not to exceed a maximum of **[****]** dollars ($**[****]**) per annum.

"GAAP" means United States generally accepted accounting principles.

"**[****]** Information" means information regarding the Warrant Holder's development of **[****]**, including information regarding available Know-How and applicable terms and conditions, including pricing.

"GLP" is defined in Section 3.6(f).

"Governmental Entity" means any instrumentality, subdivision, court, administrative agency, commission or other similar authority of any country, state, province, prefect, municipality, locality or other government or political subdivision thereof, or any quasi-governmental, private body or arbitral body exercising any executive, legislative, judicial, quasi-judicial, regulatory, taxing, importing, administrative or other governmental or quasi-governmental authority.

"Hazardous Material" means any chemical, pollutant, contaminant, pesticide, fungicide, rodenticide, poison, petroleum or petroleum product, radioactive substance, biological material, genetically modified organism, wastes (including solid, hazardous, extremely hazardous, special, dangerous, or toxic), and any substance, chemical or material regulated, listed, limited or defined as such under any Environmental Law, including: (a) any by-products, derivatives, or combinations of such material; (b) lead, asbestos, asbestos-containing material, presumed asbestos-containing material, poly-chlorinated biphenyls, solvents and waste oil, and mold or other indoor air contaminants; (c) any "hazardous substance," "pollutant," "toxic pollutant" or "contaminant" as defined under Environmental Laws; (d) any "hazardous waste" as defined under RCRA, or any Environmental Law applicable to the management of waste; and (e) any other substance which may be subject of regulatory action by any Governmental Entity in connection with any Environmental Law.

"HSR Act" means the Hart-Scott-Rodino Antitrust Improvements Act of 1976.

"IND" means an Investigational New Drug Application, as such term is defined under the FDCA and 21 CFR Part 312, or an analogous application or submission with any analogous agency or Regulatory Entity outside of the United States.

"Indebtedness" of any Person means, without duplication, (i) all indebtedness of such Person for borrowed money or indebtedness issued or incurred in substitution or exchange for indebtedness for borrowed money, (ii) all obligations of such Person with respect to deposits or advances of any kind to such Person or for the deferred purchase price of property or services (other than current trade liabilities incurred in the Ordinary Course of Business and payable in accordance with customary practices and not more than 90 days past due), (iii) all obligations of such Person evidenced by bonds, debentures, notes, mortgages or similar instruments, (iv) all obligations of such Person under conditional sale or other title retention agreements relating to any assets and properties purchased by such Person, (v) all Indebtedness of others secured by (or for which the holder of such Indebtedness has an existing right, contingent or otherwise, to be secured by) any Lien or other claim on any assets and properties owned or acquired by such Person, whether or not the obligations secured thereby have been assumed, (vi) all guarantees by such Person or conditional payment obligations of such Person with respect to the Indebtedness of others, (vii) all lease obligations of such Person required in accordance with GAAP to be recorded as capital leases, (viii) all obligations of such Person as an account party in respect of letters of credit and banker's acceptances, (ix) all obligations of such Person consisting of overdrafts (e.g., cash float reflected as a negative on the cash line), (x) all obligations of such Person pursuant to any deferred compensation agreements accrued as of the date of determination and (xi) obligations under any interest rate, currency or other hedging agreement.

"Indemnified Party" is defined in Section 7.3(a).

"Indemnifying Party" is defined in Section 7.3(a).

"Intellectual Property" means all rights, title, and interests in and to all intellectual property rights of every kind and nature however denominated, throughout the world, including:

(a) patents, copyrights, mask work rights, confidential information, trade secrets, database rights, invention disclosures and all other proprietary rights in embodiments;

(b) trademarks, trade names, service marks, service names, brands, trade dress and logos, and the goodwill and activities associated therewith;

(c) domain names and social media accounts;

(d) rights of privacy and publicity, and moral rights; and

(e) any and all registrations, applications, recordings, licenses, common-law rights, statutory rights, administrative rights, and contractual rights relating to any of the foregoing.

"IRS" means the Internal Revenue Service of the United States of America.

"Joint Patent" means a Patent claiming an invention conceived jointly by a Party's or its Affiliates' employees, agents, or independent contractors, or any persons contractually required to assign or license such invention to such Party or any Affiliate of such Party, on the one hand, and the other Party's or its Affiliates' employees, agents, or independent contractors, or any persons contractually required to assign or license such invention to such Party or any Affiliate of such Party, on the other hand, at any time from and after the License Grant Date until the end of the Warrant Period.

"Judgment" means any writ, judgment, injunction, order, decree, stipulation determination or award.

"JSC" or "Joint Steering Committee" are defined in Section 6.1(a).

"Know-How" means any proprietary information and materials, including records, discoveries, improvements, modifications, processes, techniques, methods, assays, chemical or biological materials, designs, protocols, formulas, data (including physical data, chemical data, toxicology data, animal data, raw data, clinical data, and analytical and quality control data), dosage regimens, control assays, product specifications, marketing, pricing and distribution costs, inventions, algorithms, technology, forecasts, profiles, strategies, plans, results in any form whatsoever, know-how and trade secrets (in each case, patentable, copyrightable or otherwise).

"Law" means any federal, state, territorial, foreign or local law, common law, statute, ordinance, rule, regulation or code of any Governmental Entity.

"Leased Property" is defined in Section 3.12(b).

"Legal Proceeding" means any claim, action, suit, proceeding (at law or in equity), including any litigation relating to Environmental Laws, or arbitration by or before any Governmental Entity or before any arbitrator or mediator or similar party.

"Legal Requirement" means any Law, any Judgment, any Permit or any similar provision having the force or effect of Law.

"Legal Restraint" is defined in Section 2.4(a)(v).

"Liabilities" means any and all debts, liabilities and obligations, whether matured or unmatured, accrued or fixed, liquidated or unliquidated, known or unknown, absolute or contingent, asserted or unasserted, due or to become due, determined, determinable or otherwise, and whether or not required under GAAP to be accrued on the financial statements of such Person.

"License Grant Date" is defined in Section 2.9(a).

"Licensed Intellectual Property" means all Intellectual Property licensed to the Company.

"Lien" means any lien, security interest, mortgage, pledge, levy, charge, conditional sale contract or other similar encumbrance of any kind, whether arising by Contract or by operation of Law.

"Losses" means any Actions, Liabilities, Governmental Orders, Liens, losses, damages, bonds, dues, assessments, fines, penalties, fees, costs (including costs of investigation, defense and enforcement of this Warrant), expenses or amounts paid in settlement (in each case including reasonable attorneys' and experts' fees and expenses), whether or not involving a third party claim.

"MAA" means a marketing authorization application, or successor application, submitted to the EMA to obtain marketing approval of a pharmaceutical product in the European Union.

"Manufacturing Costs" means (a) with respect to the Product that is manufactured and supplied by a Third Party on behalf of the Company, **[****]**; and (b) to the extent the Product is manufactured and supplied by a Party or its Affiliates, **[****]**; provided, that, with respect to manufacturing overhead attributable to the Product, Manufacturing Costs calculated in accordance with clause (b) shall not include **[****]**.

"Mark" means any trademark, trade name, service mark, service name, product name, brand, domain name, trade dress, logo, slogan, or other indicia of origin or ownership, and (a) all registrations, applications for registrations, and other intellectual property rights associated with any of the foregoing, and (b) the goodwill associated with each of the foregoing.

"Material Adverse Change" means any change, effect, event, occurrence or development which, individually or in the aggregate, (i) would reasonably be expected to result in, or has resulted in, any change or effect that is materially adverse to the business, assets, financial condition or operations of the Company, taken as a whole, or (ii) would reasonably be expected to prevent the consummation of the Warrant Exercise Closing or the other transactions contemplated by this Warrant; provided that none of the following shall be deemed, either alone or in combination, to constitute, and none of the following shall be taken into account in determining whether there has been or will be, a Material Adverse Change: (A) any change, effect, event, occurrence or development relating to the economy in general in the United States or in any other jurisdiction in which the Company has operations or conducts business, (B) any change, effect, event, occurrence or development reasonably attributable to conditions affecting the industry in which the Company participates (other than as may arise or result from regulatory action by a Governmental Entity), (C) any failure, in and of itself, by the Company to meet any internal projections, forecasts or revenue or earnings predictions for any period ending on or after the date of this Warrant (it being understood that the facts or occurrences giving rise to or contributing to such failure may be deemed to constitute, or be taken into account in determining whether there has been or will be, a Material Adverse Change), (D) any change, effect, event, occurrence or development relating to national or international political or social conditions, including the engagement by the United States in hostilities, whether or not pursuant to the declaration of a national emergency or war, or the occurrence of any military or terrorist attack upon the United States of America, or any of its territories, possessions, or diplomatic or consular offices or upon any military installation, equipment or personnel of the United States of America, (E) changes in Laws or accounting rules or principles, including GAAP, (F) the announcement of (1) the issuance of this Warrant or (2) the consummation of the Warrant Exercise and the other transactions contemplated by this Warrant or (G) the failure of the Company to expend more than $**[****]** in the aggregate in furtherance of the Development Program; except in the case of clauses (A), (B), (D) and (E) to the extent that any such change, effect, event, occurrence or development has or would reasonably be expected to have a disproportionate adverse effect on the business, financial condition or operations of the Company, relative to that of other pharmaceutical development companies similar to the Company.

"Material Contract" is defined in Section 3.13(a).

"Minimum" is defined in Section 7.1.

"Most Recent Balance Sheet" means the balance sheet of the Company as of the Most Recent Balance Sheet Date.

"Most Recent Balance Sheet Date" is defined in Section 3.8.

"NCH" means Nationwide Children's Hospital, an Ohio nonprofit corporation.

"NCH License Agreement" means the Exclusive License Agreement by and between the Company and NCH, dated as of May 16, 2017, as amended by a First Amendment, dated as of February 20, 2018.

"NCH Licensed Intellectual Property" means the Intellectual Property and applications for Intellectual Property licensed to the Company pursuant to the NCH License Agreement.

"NDA" means (i) a New Drug Application or Biologic License Application filed with the FDA or (ii) a MAA filed with the EMA, in each case, for marketing approval of a pharmaceutical product or any successor applications or procedures, and all supplements and amendments that may be filed with respect to the foregoing. The term "NDA" shall not include applications for pricing or reimbursement approval.

"Net PRV Proceeds" means **[****]** from the sale of a Priority Review Voucher to a Third Party as contemplated by Section 2.6(a)(ii), **[****]**.

"Net Sales" means the gross amounts invoiced on sales of a Product by the Warrant Holder or its Related Parties to a Third Party purchaser in an arms-length transaction (including to a Third Party Distributor), less the following deductions actually taken, paid, accrued, allowed, included, or allocated based on good-faith estimates in the gross sales price with respect to such sales:

(a) **[****]**;

(b) **[****]**;

(c) **[****]**;

(d) **[****]**; and

(e) **[****]**.

[****].

Notwithstanding the foregoing, the following will not be included in Net Sales: (1) sales between or among the Warrant Holder and its Related Parties, (but Net Sales shall include sales to the first Third Party (other than a licensee or sublicensee) by the Warrant Holder or a Related Party and shall also include sales to a Third Party Distributor (even if such Third Party Distributor is a licensee or sublicensee), (2) any resale of a Product by a Third Party Distributor (even if such Third Party Distributor is a licensee or sublicensee), (3) Product used as samples to promote additional Net Sales, in amounts consistent with normal business practices of the applicable Warrant Holder or Related Party, and (4) Product sales for compassionate use, "named patient sales," use under the ATU system in France or other equivalent systems, sales made in connection with Clinical Trials and product donations of a Product, in case of clauses (3) and (4), made at or below the Warrant Holder's or Related Party's, as applicable, Manufacturing Cost therefor.

Net Sales shall be accounted for in accordance with GAAP, consistently applied. Any price discounts offered by Warrant Holder or a Related Party to purchasers of a Product will not exceed in the aggregate the discount levels customary in the industry for products that are comparable to a Product at a similar stage in the product life cycle.

In the event that a Product is sold in the form of a combination product containing one or more active pharmaceutical ingredients in addition to such Product, Net Sales of such combination product shall be adjusted by multiplying the actual Net Sales (as defined above) for such combination product by the fraction A/(A+B) where A is the average sale price of the Product when sold separately and B is the total of the average sale prices of the other active pharmaceutical ingredient(s) when sold separately, in each case, during the applicable Quarter in the country in which the sale of the combination product occurred, or if the sales of both the Product, on the one hand, and the other active pharmaceutical ingredients, on the other hand, did not occur in such country in such period, then in the most recent Quarter in which all such sales occurred. Alternatively, in the event that such average sale prices cannot be determined for both the Product, on the one hand, and all other active pharmaceutical ingredients included in the combination product, on the other hand, then Net Sales shall be adjusted by **[****]**.

"Note Conversion Agreement" means the Note Conversion Agreement, dated as of May 2, 2018, by and among the Company and the Convertible Noteholders.

"Ordinary Course of Business" means the ordinary course of business, consistent with any past practice or, with respect to matters covered under the Development Plan and Budget, materially in accordance with the Development Plan and Budget.

"Owned Intellectual Property" means all Intellectual Property solely or jointly owned by the Company.

"Party" means the Warrant Holder or the Company (or, after the Warrant Exercise Closing Date, the Stockholder Representative), as the case may be.

"Patent" means any United States or foreign patent, any application for a United States or foreign patent, or any continuation, division or reissue thereof.

"Permit" means any federal, state or local, domestic or foreign governmental consent, approval, order, authorization, permit, concession, registration, franchise, license or similar right.

"Permitted Liens" means the following: (i) statutory Liens for Taxes not yet due or payable; (ii) Liens for assessments and other governmental charges or Liens of landlords, carriers, warehousemen, mechanics and repairmen incurred in the Ordinary Course of Business, in each case, for sums not yet due and payable or due but not delinquent or being contested in good faith by appropriate proceedings; (iii) Liens incurred in the Ordinary Course of Business in connection with workers' compensation, unemployment insurance and other types of social security; and (iv) encumbrances in the nature of zoning restrictions, easements, rights or restrictions of record on the use of real property if the same do not materially detract from the value of the property encumbered thereby or materially impair the use of such property in the Company's business.

"Person" means an individual, corporation, company, partnership, limited liability company, joint venture, association, trust, business trust, unincorporated organization or any other entity or organization, including a Governmental Entity.

"Personal Property Leases" is defined in Section 3.11(b).

"Plan" is defined in Section 3.19(a).

"Post-Closing Tax Period" means any Tax period beginning after the date of the Warrant Exercise Closing and that portion of any Straddle Period beginning on the day after the date of the Warrant Exercise Closing Date (as determined in accordance with Section 5.5(g)(viii)).

"PPACA" is defined in Section 3.18(g).

"Pre-Closing Tax Liabilities" means all Tax Liabilities of the Company for all Pre-Closing Tax Periods, determined without taking into account any Tax refunds or other Tax assets.

"Pre-Closing Tax Period" means any Tax period ending on or before the date of the Warrant Exercise Closing and that portion of any Straddle Period ending on the date of the Warrant Exercise Closing (as determined in accordance with Section 5.5(g)(viii)).

"Priority Review Voucher" means a priority review voucher granted by the FDA solely with respect to any Product Candidate and not with respect to any other product candidate of the Warrant Holder.

"Priority Review Voucher Deal Fees" means all fees and expenses (including fees and expenses of investment bankers, finders, consultants, attorneys, accountants or others) of the Warrant Holder or the Company reasonably incurred or reimbursable by the Warrant Holder or the Company in connection with the negotiation, entering into and consummation of the sale of a Priority Review Voucher.

"Product" shall mean a product containing a Product Candidate.

"Product Candidate" means each of MYO-101, MYO-102, MYO-103, MYO-201 and MYO-301, as defined on Exhibit B, in each case in any form or formulation.

"Property Taxes" is defined in Section 5.5(g)(viii)(A).

"PTO" is defined in Section 3.14(c).

"PTO Filings" is defined in Section 5.8.

"Public Official" means (i) any officer, employee or representative of any Governmental Entity; (ii) any officer, employee or representative of any commercial enterprise that is owned or controlled by a Governmental Entity, including any state-owned or controlled veterinary or medical facility; (iii) any officer, employee or representative of any public international organization, such as the African Union, the International Monetary Fund, the United Nations or the World Bank; (iv) any Person acting in an official capacity for any Governmental Entity, enterprise, or organization identified above; and (v) any political party, party official or known candidate for political office.

"Purchase Amount" means the number equal to the product of (i) **[****]** multiplied by (ii) the number of shares of Company Capital Stock issued and outstanding immediately prior to the Warrant Exercise Closing on a fully-diluted basis, as if all such shares then convertible into or exercisable for shares of Company Common Stock were so converted or exercised immediately prior to such calculation.

"Qualifying Acquisition Proposal" means any offer or proposal from any Person relating to any (i) direct or indirect acquisition or sale of all or substantially all of the assets of the Company or (ii) direct or indirect acquisition of a majority of the Capital Stock of the Company (whether through a share purchase, merger, consolidation, business combination, recapitalization or similar transaction involving the Company), in each case that (x) does not provide the Company or the Company Equityholders with consideration greater than the Warrant Exercise Payment and (y) that the Board of Directors of the Company, determines, in its sole discretion, constitutes an acceptable offer or proposal for the assets or Capital Stock, as applicable, and in the case of each of (i) and (ii) other than (A) the Warrant Exercise Closing and the other transactions contemplated by this Warrant or (B) **[****]**.

"Qualifying Licensing Proposal" means any offer or proposal from any Person for a license to use, develop or commercialize any Product Candidate or any of the Company Intellectual Property Covering or otherwise relating to the Product Candidate that (x) does not provide the Company or the Company Equityholders with consideration greater than the Warrant Exercise Payment and (y) that the Board of Directors of the Company, determines, in its sole discretion, constitutes an acceptable offer or proposal for such licensing arrangement.

"Quarter" means a period of three (3) consecutive months ending on the last day of March, June, September, or December, respectively.

"RCRA" means the Resource Conservation and Recovery Act (42 U.S.C. §§ 6901 et seq.), as amended, and any foreign and state Law counterparts.

"R&W Insurance Policy" is defined in Section 5.24.

"R&W Insurance Policy Cost" mean the amount equal to the aggregate amount of the premium, underwriting fee, brokerage fees, legal fees (if any) for counsel engaged by the underwriter, surplus lines tax and any other costs and expenses associated with obtaining the R&W Insurance Policy, limited in the case of the premium to the amount that would have been due had the policy limit been $**[****]** in the case the Warrant Holder elects to purchase a policy with a higher policy limit.

"Redemption Provisions" means Article V, Section C of the Certificate of Incorporation.

"Regulatory Entity" means the FDA, the EMA, or any other national, supra-national, regional, state or local regulatory agency, department, bureau, commission, council or other Governmental Entity involved in the granting of any approval required by applicable Laws to promote, market and sell pharmaceutical products.

"Regulatory Submission" is defined in Section 3.6(c).

"Related Party" means a Party's Affiliates, licensees and sublicensees but, with respect to the Warrant Holder, excluding Third Party Distributors. Related Party shall also include any subsequent assignee or transferee of the Product or Company Patents.

"Release" means any spill, discharge, leak, migration, emission, escape, injection, dumping, leaching, or other release of any Hazardous Material into the indoor or outdoor environment, whether or not intentional, and whether or not notification or reporting to any Governmental Entity was or is required at the time it initially occurred or continued to occur. Without limiting the above, Release includes the meaning of "Release" as defined under CERCLA.

"Representatives" means with respect to a Person, such Person's legal, financial, internal and independent accounting and other advisors and representatives.

"Restricted Stock" means any Company Capital Stock that is subject to a right of repurchase or redemption (other than pursuant to the Redemption Provisions) by the Company or subject to forfeiture back to the Company.

"Review Period" is defined in Section 2.3(a).

"Securities Act" means the Securities Act of 1933, or any successor federal statute, and the rules and regulations of the Commission thereunder, as amended from time to time.

"Shares" means the Company's authorized Common Stock, $0.0001 par value per share.

"Stockholder Acknowledgement" has the meaning ascribed to it in the Certificate of Incorporation.

"Stockholder Representative Agreement" means the Stockholder Representative Agreement to be entered into by the Company and the Company Stockholders prior to the Warrant Exercise Closing Date.

"Stockholder Representative" means the Person appointed by the Company Stockholders (or the Company, as applicable) to serve as the agent, representative and attorney-in-fact of the Company Stockholders or appointed as provided in Section 5.23.

"Stockholder Representative Reserve" means an amount specified by the Company at the time the Company notifies the Warrant Holder of the appointment of the Stockholder Representative pursuant to the Section 5.23 (or, if the Stockholder Representative is appointed by a court of competent jurisdiction pursuant to Section 5.23, an amount specified by such court).

"Straddle Period" is defined in Section 5.5(g).

"Subsidiary" means, with respect to any Person, (a) any corporation more than fifty percent (50%) of whose stock is owned by such Person directly or indirectly through one or more Subsidiaries of such Person and (b) any partnership, association, joint venture or other entity in which such Person directly or indirectly through one or more Subsidiaries of such Person has more than a fifty percent (50%) equity interest.

"Successful Biopsy Analysis" means the collection of a tissue sample suitable for analysis together with an assay performed that demonstrates measurement of protein restoration using validated methods.

"Target Warrant Exercise Closing Date" is defined in Section 2.3(a).

Tax" (and, with correlative meaning, "Taxes" and "Taxable") means (a) any United States local, state or federal or foreign taxes, including income, capital gains, alternative or add-on minimum, estimated, gross income, gross receipts, sales, use, value added, ad valorem, franchise, capital stock or other equity securities, profits, license, registration, withholding, employment, unemployment, disability, severance, occupation, social security (or similar including FICA), payroll, transfer, conveyance, documentary, stamp, property (real, tangible or intangible), premium, escheat or unclaimed property obligation, environmental, windfall profits, customs duties, net proceeds, goods and services, leasing, registration or other taxes of any kind or any fees, charges, levies, excises, duties or assessments of any kind in the nature of (or similar to) taxes whatsoever, and including any addition to tax or additional amount, together with any interest, penalties or addition thereto, whether disputed or not, and (b) any Liability for the payment of any amount of any type described in clause (a) of this sentence as a result of being or having been a member of an member of an affiliated, consolidated, combined, unitary or aggregate group for any Tax period, and (c) any Liability for the payment of any amounts of the type described in clause (a) or (b) of this sentence as a result of being a transferee of or successor to any Person or as a result of any express or implied obligation to assume such Taxes or to indemnify any other Person, by Contract or otherwise.

"Tax Law" means all currently applicable Laws relating to or regulating the assessment, determination, collection or imposition of Taxes, including any formal or informal interpretation or guidance issued by a Taxing Authority.

"Tax Return" means any report, return, declaration, claim for refund, information return, statement, designation, election, notice or certificate filed or required to be filed with any Taxing Authority, including any schedule or attachment thereto and including any amendment thereof.

"Taxing Authority" means any Governmental Entity having jurisdiction over the assessment, determination, collection, or imposition of any Taxes (domestic or foreign).

"Third Party" shall mean any Person other than the Warrant Holder of the Company or their respective Affiliates.

"Third Party Claim" means any suit, proceeding, claim or demand by a Person other than a Person from which indemnification may be sought under Article 7.

"Third Party Distributor" means any Third Party that purchases Products from the Warrant Holder or any Related Parties and distributes such Product directly to customers, but does not develop or manufacture any Product and does not make any royalty, profit-share or other payment to the Warrant Holder or any Related Parties, other than payment for the purchase of Products for resale.

"Third Party Transaction Notice" is defined in Section 2.8(b).

"Threshold" is defined in Section 7.1.

"Topping Offer" is defined in Section 2.8(b).

"Topping Period" is defined in Section 2.8(b).

"Transaction Proposal" means any inquiry, proposal or offer from any Person relating to, or that would reasonably be expected to lead to, any (i) direct or indirect acquisition or sale of substantially all of the assets of the Company, (ii) license or grant of rights to any Collaboration Party to use, develop or commercialize a Product Candidate or any of the Company Intellectual Property, or (iii) direct or indirect acquisition of a majority of the Capital Stock of the Company (whether through a share purchase, merger, consolidation, business combination, recapitalization or similar transaction involving the Company), in each case that the Board of Directors of the Company, determines, in its sole discretion and in accordance with its fiduciary duties, is an acceptable offer for the assets or Capital Stock, as applicable, and in each case other than (A) the Warrant Exercise Closing and the other transactions contemplated by this Warrant or (B) **[****]**.

"Transfer Taxes" means all transfer, sales, use, registration, real property transfer, goods and services, documentary or mortgage recording, value added, stamp and similar Taxes and fees (including any penalties and interest) incurred, imposed, assessed or payable in connection with or as a result of the transactions contemplated in the Redemption Provisions or this Warrant.

"Treasury Regulations" means the final or temporary regulations promulgated by the United States Department of the Treasury pursuant to the Code.

"Trigger Event" means: (a) completion of a Successful Biopsy Analysis on each of the patients within cohort 2 capable of providing a biopsy at the end of the 60-day period (60-day biopsy) of the Company Phase 1/2A Clinical Trial of MYO-101, and (b) delivery by the Company to the Warrant Holder of a cohort 2 biopsy results report **[****]**.

"Triggering Data" means the cohort 2 biopsy results report **[****]**.

"WARN" is defined in Section 3.19(d).

"Warrant" means, as the context requires, this Share purchase warrant and any successor warrant or warrants issued upon a permitted transfer or assignment of this Share purchase warrant or of any such successor warrant.

"Warrant Exercise" is defined in Section 2.4(a).

"Warrant Exercise Closing" is defined in Section 2.4(a).

"Warrant Exercise Closing Date" means the date on which the Warrant Exercise Closing occurs.

"Warrant Exercise Payment" means **[****]**.

"Warrant Expiration Date" means the final day of the Review Period.

"Warrant Grant Payment" means an amount equal to $60,000,000.

"Warrant Holder" is defined in the Preamble.

"Warrant Holder Disclosure Schedule" means a schedule of exceptions to the representations and warranties of the Warrant Holder set forth in Article 4. The Warrant Holder Disclosure Schedule shall be arranged in sections and subsections corresponding to the numbered and lettered sections and subsections contained in Article 4. The disclosures in any section or subsection of the Warrant Holder Disclosure Schedule shall be deemed disclosure with respect to any other section or subsection of the Warrant Holder Disclosure Schedule to which the relevance of such item is reasonably apparent on the face of that disclosure.

"Warrant Holder Indemnified Party" is defined in Section 7.1(a).

"Warrant Period" means the period commencing on the date hereof and ending on the earlier to occur of the date of the Warrant Exercise Closing Date or the date of termination of this Warrant in accordance with Section 8.1.

"Warrant Shares" means the number of Shares issued or issuable upon exercise of this Warrant as set forth in the introduction hereto, as adjusted from time to time to account for any reclassifications, exchange, substitution, payment of a dividend in securities or property or other event that results in a change of the number and/or class of the securities issuable upon exercise or conversion of this Warrant.

Section 1.2. Rules of Construction and Interpretation.

(a) Construction. Except where expressly stated otherwise in this Warrant, the following rules of interpretation apply to this Warrant:

(i) "or" has the inclusive meaning represented by the phrase "and/or";

(ii) "include", "includes" and "including" are not limiting;

(iii) "hereof", "hereto", "hereby", "herein" and "hereunder" and words of similar import when used in this Warrant refer to this Warrant as a whole and not to any particular provision of this Warrant;

(iv) "date hereof" refers to the date of this Warrant;

(v) "extent" in the phrase "to the extent" means the degree to which a subject or other thing extends, and such phrase does not mean simply "if";

(vi) definitions contained in this Warrant are applicable to the singular as well as the plural forms of such terms;

(vii) references to an agreement or instrument mean such agreement or instrument as from time to time amended, modified or supplemented;

(viii) references to a Person are also to its permitted successors and assigns;

(ix) references to an "Article", "Section", "Subsection", "Exhibit" or "Schedule" refer to an Article of, a Section or Subsection of, or an Exhibit or Schedule to, this Warrant;

(x) words importing the masculine gender include the feminine or neuter and, in each case, *vice versa*;

(xi) "day" or "days" refers to calendar days;

(xii) references to a Law include any amendment or modification to such Law and any rules or regulations issued thereunder, whether such amendment or modification is made, or issuance of such rules or regulations occurs, before or, only with respect to events or developments occurring or actions taken or conditions existing after the date of such amendment, modification or issuance, after the date of this Warrant, but only to the extent such amendment or modification, to the extent it occurs after the date hereof, does not have a retroactive effect.

(b) Accounting Terms and Determinations. Unless otherwise specified herein, all accounting terms used herein shall be interpreted and all accounting determinations hereunder shall be made in accordance with GAAP. References to fiscal periods are to fiscal periods of the Company.

(c) Exhibits and Schedules. All of the exhibits and schedules attached hereto shall be deemed incorporated herein by reference.

(d) No Presumption Against Any Party. Neither this Warrant nor any uncertainty or ambiguity herein shall be construed or resolved using any presumption against any party hereto, whether under any rule of construction or otherwise. On the contrary, this Warrant has been reviewed by each of the parties and their counsel and, in the case of any ambiguity or uncertainty, shall be construed and interpreted according to the ordinary meaning of the words used so as to fairly accomplish the purposes and intentions of all parties hereto.

(e) Section Headings. The section headings used herein are for convenience of reference only and shall not be construed in any way to affect the interpretation of any provisions of the Warrant.

ARTICLE 2
WARRANT PAYMENT AND EXERCISE

Section 2.1. Warrant Payments Prior to Warrant Exercise.

(a) Grant of Warrant. As consideration for the issuance of this Warrant, within three (3) Business Days after the date hereof, the Warrant Holder shall pay to the Company, in cash, the Warrant Grant Payment. The obligation of the Warrant Holder to pay the Warrant Grant Payment shall be contingent upon the receipt by the Warrant Holder of the following documents and items on or before the date this Warrant is executed by both Parties:

(i) Evidence of the acceptance by the Secretary of State of the State of Delaware of the filed Certificate of Incorporation in the form of Exhibit C attached hereto;

(ii) A copy of the Company's 2017 Equity Incentive Plan, as duly adopted by the Board of Directors of the Company;

(iii) A fully executed copy of the Note Conversion Agreement, in the form of Exhibit D attached hereto, by and among the Company and each Convertible Noteholder;

(iv) A fully executed copy of a Consent Agreement, in the form of Exhibit E attached hereto, by and among the Company and each holder of Company Capital Stock as of the date hereof; and

(v) One uncertificated share of Class G Preferred Stock, representing all outstanding shares of Class G Preferred Stock, issued to the Warrant Holder.

(b) Development Milestone Events.

(i) Provided that this Warrant has not terminated effective prior thereto, upon achievement of each Development Milestone Event the Company shall promptly deliver to the Warrant Holder (and in any event, no later than **[****]** days following such Development Milestone Event) updated Disclosure Schedules (if required by Section 2.2(a)) and a written certification, executed by the Chief Executive Officer, certifying that such Development Milestone Event has occurred (a "Development Milestone Event Notice").

(ii) During each Development Milestone Review Period the Company shall use Commercially Reasonable Efforts to respond to any diligence requests made by the Warrant Holder or its Representatives as promptly as practicable and in any event reasonably in advance of the end of such Development Milestone Event.

(iii) If (A) no event enumerated in Section 2.1(d) has occurred or is occurring and (B) the conditions in Section 2.1(c) have been satisfied, the Warrant Holder shall pay the applicable Development Milestone Payment to the Company on or prior to the last day of the applicable Development Milestone Review Period. This clause (iii) shall apply only to the first achievement of a Development Milestone Event, such that each Development Milestone Payment shall not be payable more than once.

(c) Development Milestone Payment Conditions. The obligation of the Warrant Holder to make each Development Milestone Payment shall be subject to the following conditions:

(i) the Warrant Holder has received the applicable Development Milestone Event Notice required by Section 2.1(b)(i) and the updated Disclosure Schedules if required by Section 2.2(a);

(ii) the Company has performed and complied in all material respects with all agreements and covenants required by this Warrant to be performed or complied with, with the exception of any breach of which the Warrant Holder has received written notice that has been cured to the Warrant Holder's reasonable satisfaction;

(iii) there is no (a) Legal Restraint which would prohibit the Warrant Exercise Closing or any other actions to be taken pursuant to the Redemption Provisions in connection with the Warrant Exercise Closing or (b) Legal Proceeding that is pending or, to the knowledge of the Company, threatened that could result in a Legal Restraint which could prohibit the Warrant Exercise Closing or any other actions to be taken pursuant to the Redemption Provisions in connection with the Warrant Exercise Closing;

(iv) between (1) the later of (X) the date of this Warrant and (2) the immediately preceding Development Milestone Event and (Y) the date of the certificate required by (vi) of this Section 2.1(c), there has been no Material Adverse Change; and

(v) the Company has delivered to the Warrant Holder a written certificate, executed by the Chief Executive Officer, certifying that, to the best of such officer's knowledge, each of the conditions set forth of in (i) through (v) of this Section 2.1(c) has been satisfied (or, in the case where any has not been satisfied, a description in reasonable detail of the reason for such non-satisfaction).

(d) With reference to Section 2.1(b)(iii), following are the referenced events: the Company (1) applies for or consents to the appointment of a receiver, trustee, custodian or liquidator of itself or substantially all of its property, (2) becomes subject to the appointment of a receiver, trustee, custodian or liquidator for itself or substantially all of its property, (3) makes an assignment for the benefit of creditors, (4) institutes any proceedings under the United States Bankruptcy Code or any other federal or state bankruptcy, reorganization, receivership, insolvency or other similar law affecting the rights of creditors generally, or files a petition or answer seeking reorganization or an arrangement with creditors to take advantage of any insolvency law, or files an answer admitting the material allegations of a bankruptcy, reorganization or insolvency petition filed against it, or (5) becomes subject to any involuntary proceedings under the United States Bankruptcy Code or any other federal or state bankruptcy, reorganization, receivership, insolvency or other similar law affecting the rights of creditors generally, which proceeding is not dismissed within thirty (30) days of filing, or have an order for relief entered against it in any proceeding under the United States Bankruptcy Code.

Section 2.2. Disclosure Schedules.

(a) No later than **[****]** days following each Development Milestone Event, the Company shall deliver to the Warrant Holder an updated Disclosure Schedule, to be dated as of the date of delivery; provided, that the Company shall not be obligated to deliver updated Disclosure Schedules to the Warrant Holder pursuant to this Section 2.2(a): (i) for any Development Milestone Event achieved prior to the first anniversary of this Warrant, and (ii) more than **[****]** in any **[****]**-month period thereafter. Such updated Disclosure Schedule shall include all disclosure necessary to make the representations and warranties of the Company set forth in this Warrant true and correct as though made as of the date of delivery, except that the accuracy of representations and warranties that by their terms speak as of a specified date will be determined as of such date. Any amendment or supplement included in such updated Disclosure Schedule shall not cure any breach or inaccuracy of any representation or warranty made as of a date prior to the delivery of such updated Disclosure Schedule pursuant to this Warrant, but, with respect to amendments or supplements to disclose information regarding events occurring after the date of receipt of the most recently delivered prior Disclosure Schedule, such updated Disclosure Schedule shall be deemed to qualify the representations and warranties contained in Article 3 as made as of the date of delivery of such updated Disclosure Schedule.

(b) Within **[****]** days following the date the Company delivers the Data Trigger Notification to the Warrant Holder, the Company shall deliver to the Warrant Holder a draft of an updated Disclosure Schedule. Such draft of an updated Disclosure Schedule shall include all disclosure necessary to make the representations and warranties of the Company set forth in this Warrant true and correct as though made as of the date of delivery of the draft updated Disclosure Schedule, except that the disclosure with respect to representations and warranties that by their terms speak as of a specified date will be the disclosure necessary to make such representations and warranties true and correct as of such date. During the period starting on the date the Company delivers the Data Trigger Notification to the Warrant Holder and through the end of the Review Period, the Warrant Holder may provide a diligence request list to the Company in order to conduct customary due diligence for the acquisition of a business, and the Company shall use Commercially Reasonable Efforts to respond to any such diligence request by providing the requested materials and information to the Warrant Holder to the extent available. The Company shall use Commercially Reasonable Efforts to answer any diligence requests as promptly as practicable.

(c) If the Warrant Holder delivers an Exercise Notice in accordance with Section 2.3(a), the Company shall deliver an updated Disclosure Schedule to the Warrant Holder at least **[****]** Business Days prior to the anticipated Warrant Exercise Closing Date, dated as of the anticipated Warrant Exercise Closing Date. Between such date of delivery and the anticipated Warrant Exercise Closing Date (as it may be extended pursuant to this Warrant), the Company may deliver to the Warrant Holder additional updated Disclosure Schedules, each dated as of the date of the anticipated Warrant Exercise Closing Date, that shall include all disclosure necessary to make the representations and warranties of the Company set forth in this Warrant true and correct as though made as of the Warrant Exercise Closing Date, except that the accuracy of representations and warranties that by their terms speak as of a specified date

will be determined as of such date. Any amendment or supplement included in such updated Disclosure Schedule shall not cure any breach or inaccuracy of any representation or warranty made as of a date prior to the delivery of such updated Disclosure Schedule pursuant to this Warrant, but, with respect to amendments or supplements to disclose information regarding events occurring after the date of the most recent prior updated Disclosure Schedule delivered pursuant to Section 2.3(a) (or, if no updated Disclosure Schedule has been delivered pursuant to Section 2.3(a), after the date of this Warrant), such updated Disclosure Schedule shall be deemed to qualify the representations and warranties contained in Article 3 as made as of the date of such updated Disclosure Schedule.

(d) In the event that any updated Disclosure Schedule that is delivered in accordance with Section 2.2(c) contains information that has not previously been disclosed to the Warrant Holder and that materially modifies the applicable representation and warranty (as modified by the previous Disclosure Schedule), the Warrant Holder may cause the Warrant Exercise Closing Date to be delayed for a reasonable period of time not to exceed **[****]** days and may request additional diligence materials and information related to the information that was not previously disclosed to the Warrant Holder, and the Company shall use Commercially Reasonable Efforts to respond to any such diligence request promptly and to provide the requested materials and information to the Warrant Holder, to the extent available, within **[****]** Business Days of such request by the Warrant Holder. Any failure to so provide such materials and information, if reasonably available, shall toll the time period referred to in the previous sentence until such materials and information are provided or the Company certifies to the Warrant Holder that no additional information is reasonably available. The Company may continue to provide updated Disclosure Schedules in accordance with Section 2.2(a) such that the most recently delivered Disclosure Schedule is accurate as of the Warrant Exercise Closing Date, as so extended, and with respect to any such updated Disclosure Schedules, the provisions of the first sentence of this Section 2.2(d) shall apply again, and the Warrant Exercise Closing Date may be extended for additional periods of time in accordance therewith.

(e) In connection with delivery of any updated Disclosure Schedule, the Company shall simultaneously deliver to the Warrant Holder copies of all Contracts or other documents disclosed in the updated Disclosure Schedule that have not been included in a previous Disclosure Schedule or previously provided to the Warrant Holder.

(f) Any updated Disclosure Schedule shall be prepared in a form and manner reasonably consistent with the Disclosure Schedule delivered to the Warrant Holder on the date of this Warrant, and reasonably sufficient to put the Warrant Holder on notice of the information being disclosed with reasonable specificity.

Section 2.3. Exercise Notice.

(a) At any time during the period commencing on the date the Company receives the Warrant Grant Payment and continuing until 11:59 pm Eastern Standard Time on the sixtieth (60th) day after the date the Warrant Holder receives the Data Trigger Notification from the Company (as such period may be extended pursuant to Section 2.2(d), the "Review Period"), the Warrant Holder may (in its sole discretion), but shall not be obligated to, deliver to

the Company written notice (an "Exercise Notice") that it intends to exercise this Warrant and the anticipated Warrant Exercise Closing Date (the "Target Warrant Exercise Closing Date'), which date may not be later than **[****]** days after the date the Exercise Notice is given. Following the Warrant Holder's delivery of an Exercise Notice to the Company, the Company and the Warrant Holder shall promptly take all actions set forth in Section 5.16 and Section 5.18. In the event that the Review Period is extended pursuant to Section 2.2(d), the Target Warrant Exercise Closing Date set forth in the Exercise Notice shall automatically be extended for an amount of days equal to the amount of days that the Review Period is extended pursuant to Section 2.2(d).

(b) Notwithstanding anything to the contrary contained herein, in the event that the conditions set forth in Section 2.4(a) have not been satisfied by the Target Warrant Exercise Closing Date specified in the Exercise Notice, the Target Warrant Exercise Closing Date shall automatically be extended for a period of **[****]** Business Days from and after the date that the conditions in Section 2.4(a) have been satisfied.

(c) None of the Warrant Holder's failure to deliver an Exercise Notice or failure to consummate the Warrant Exercise Closing (if no Exercise Notice has been given or, if an Exercise Notice has been given, if the conditions set forth in Section 2.4(a) are not satisfied prior to termination of this Warrant), shall, in and as of itself, result in any Liability of the Warrant Holder to the Company or to the holders of Company Stock Options, Company Warrants or Company Capital Stock, for any reason.

Section 2.4. Warrant Exercise.

(a) If the Warrant Holder has delivered an Exercise Notice in accordance with Section 2.3(a) (the "Warrant Exercise"), the closing of the Warrant Exercise (the "Warrant Exercise Closing") shall be held on the Target Warrant Exercise Closing Date specified in the Exercise Notice (as such date may be extended pursuant to Section 2.3(a) or Section 2.3(b)), or such other date as the Parties may mutually agree, subject to the satisfaction of the following conditions (other than those conditions that may only be satisfied on the Warrant Exercise Closing Date, but subject to the satisfaction of such conditions on the Warrant Exercise Closing Date), unless otherwise waived:

(i) Each of the representations and warranties of the Company contained in this Warrant shall be true and correct as of the Warrant Exercise Closing Date with the same force and effect as if made at and as of the Warrant Exercise Closing Date (other than those representations and warranties that address matters only as of a particular date or only with respect to a specific period of time, which need only be true and correct as of such date or with respect to such period), in each case except inaccuracies that would not constitute a Material Adverse Change;

(ii) The Company shall have performed or complied in all material respects with all agreements and covenants required by this Warrant to be performed or complied with by it at or prior to the Warrant Exercise Closing Date;

(iii) All consents of Governmental Entities required in connection with the Warrant Exercise Closing and any other actions contemplated by this Warrant and the Certificate of Incorporation in connection with the Warrant Exercise Closing, shall have been obtained or made, and shall be in full force and effect;

(iv) The Company shall have obtained all consents, assignments and approvals of third parties set forth in Section 3.13(b) of the Disclosure Schedule (other than any such consent, assignment or approval under a Contract that has terminated prior to the date of the Warrant Exercise Closing) and any other consents, assignments, approvals or waivers required to be obtained by the Company with respect to the transactions contemplated hereby, if any, as set forth on Section 2.4(a) of the Disclosure Schedules;

(v) No temporary restraining order, preliminary or permanent injunction or other order or decree issued by any court of competent jurisdiction or other legal restraint, Law, or prohibition (collectively, "Legal Restraints") which prohibits the Warrant Exercise Closing or any actions to be taken pursuant to the Redemption Provisions in connection with the Warrant Exercise Closing shall be in effect;

(vi) There are no Legal Proceedings seeking any prohibition, limitation or other requirement of the type set forth in clauses (ii) through (iv) of Section 5.18(b);

(vii) The Company shall have delivered to the Warrant Holder a certificate in the form attached as Exhibit F, dated the date of the Warrant Exercise Closing Date, executed by the Chief Executive Officer of the Company, certifying as to as to the accuracy of the Company's representations and warranties, compliance as to with covenants and other obligations and the lack of any Material Adverse Change since the later of (1) the date of this Warrant and (2) the most recently delivered certificate pursuant to Section 2.1(c)(v); and

(viii) The Company shall have delivered to the Warrant Holder the deliverables listed in Section 2.4(b).

Neither Party may rely, either as a basis for not consummating the Warrant Exercise Closing or terminating this Warrant and abandoning the transactions contemplated, on the failure of any condition set forth in this Section 2.4(a) to be satisfied if such failure was caused by such Party's material breach of any provision of this Warrant.

(b) Company Deliveries. At the Warrant Exercise Closing, the Company shall execute and deliver the following to the Warrant Holder:

(i) Share Certificate. The Company shall deliver a certificate or certificates representing the number of Warrant Shares, issued in the name of the Warrant Holder or in such other name or names of any Person or Persons designated by the Warrant Holder.

(ii) FIRPTA Certificates. The Company shall deliver a duly executed certificate, in a form reasonably acceptable to the Warrant Holder, dated as of the date of the Warrant Exercise Closing pursuant to Treasury Regulations Section 1.897-2(h) (as described in Treasury Regulations Section 1.1445-2(c)(3)) stating that the Company is not, and has not been during the relevant period specified in Section 897(c)(1)(A)(ii) of the Code, a United States real property holding corporation as defined in Section 897 of the Code, together with notice to the IRS as described in Treasury Regulations Section 1.897-2(h)(2).

(iii) 280G Approval. Evidence to the reasonable satisfaction of the Warrant Holder that the Company has complied with Section 5.5(f).

(iv) Termination of Company Stock Plan and Company Stock Options. Evidence to the satisfaction of the Warrant Holder that on and after the Warrant Exercise Closing, no Person, including any current or former employee or other service provider (or their beneficiaries), will have any right to receive shares of Company Capital Stock upon the exercise of any Company Stock Option or otherwise, except as may have been consented to in writing by the Warrant Holder.

Section 2.5. Payments Upon Warrant Exercise Closing; Automatic Issuance of Shares Upon Payment

. At the Warrant Exercise Closing, the Warrant Holder shall pay (a) the Warrant Exercise Payment to the Company, and (b) the Stockholder Representative Reserve Amount to the Company. Immediately upon receipt by the Company of the Warrant Exercise Payment, the Warrant Shares shall automatically, without any further action needing to be taken on the part of the Company, be issued to the Warrant Holder, and at such time the Warrant Holder will, for all purposes, become the holder of record of the Warrant Shares.

Section 2.6. Warrant Payments After Exercise.

(a) In the event the Warrant Holder exercises the Warrant and acquires the Warrant Shares, the Warrant Holder shall make the payments described below (the "Contingent Payments") by making a payment in cash to the Company and by causing the Company then to pay such amounts to the Company Equityholders in accordance with the Redemption Provisions upon the occurrence of the following events (each, a "Contingent Payment Event"):

(i) Upon reaching cumulative Net Sales in excess of $**[****]** (the "Sales Milestone Event"), the Warrant Holder shall make a one-time Contingent Payment to the Company in an aggregate amount equal to $**[****]**; and

(ii) If one or more Priority Review Vouchers has been granted with respect to any Product Candidate, upon the sale by the Company or an Affiliate of each such Priority Review Voucher to a Third Party, the Warrant Holder shall make a Contingent Payment to the Company, and shall cause the Company then to pay such amounts to the Company Equityholders pursuant to the Redemption Provisions, in an aggregate amount equal to **[****]**; provided, that the aggregate of all such Contingent Payments from the sale of Priority Review Vouchers shall not exceed $**[****]**.

(b) The Contingent Payment set forth in Section 2.6(a)(i) is only payable once regardless of the number of different Products, Product Candidates or indications with respect to which any given Contingent Payment Event is achieved.

(c) Within **[****]** days after the public disclosure of the Warrant Holder's financial results of operations for the quarter in which the Sales Milestone Event is achieved or within **[****]** days after the occurrence of the consummation of the sale of a Priority Review Voucher, the Warrant Holder shall provide written notice to the Stockholder Representative that such Contingent Payment Event has occurred and shall pay, or cause to be paid, the applicable Contingent Payment in accordance with Section 2.6(a). Each such written notice shall be accompanied by a statement signed by an executive officer of the Warrant Holder setting forth the calculation of each Contingent Payment in reasonable detail.

(d) The Company acknowledges and agrees that after the Warrant is exercised (i) the Warrant Holder has total and absolute discretion in the exploitation of, and operation of its business and the development and commercialization of any Product Candidate, (ii) the Contingent Payments are contingent upon satisfaction of events that may not occur and may therefore never be paid, (iii) the Warrant Holder has made no representation or warranty to the Company that the conditions to any such payments will be satisfied, (iv) the Company has not relied on any such statement by the Warrant Holder or any Third Party and (v) the Warrant Holder is under no obligation to use any standard of diligence with respect to satisfying the conditions to any payment hereunder, and the Company hereby waives any such potential other standard of diligence.

(e) Before signing this Warrant the Parties have had numerous conversations and have generated correspondence and other writings, in which the Parties discussed the transaction which is the subject of this Warrant and their aspirations for success. In such conversations and writings, the Parties and individuals representing them may have expressed their judgments and beliefs concerning the intentions, capabilities, and practices of the Parties, and may have forecasted future events. The Parties recognize that such conversations and writings often involve an effort by both sides to be positive and optimistic about the prospects for the transaction. However, each Party acknowledges that all business transactions contain an element of risk, as do the transactions contemplated by this Warrant, and that it is normal business practice to limit the legal obligations of contracting Parties to only those promises and representations which are essential to their transaction so as to provide certainty as to their respective future rights and remedies. Accordingly, other than the Confidentiality Agreement, this Warrant is intended to define the full extent of the legally enforceable undertakings of the Parties, and no promise or representation, written or oral, which is not set forth explicitly in this Warrant or such Confidentiality Agreement is intended by either Party to be legally binding. Each of the Parties acknowledge that in deciding to enter into this Warrant and to consummate the transaction contemplated hereby none of them has relied upon any statements or representations, written or oral, other than those explicitly set forth in this Warrant or in the Confidentiality Agreement.

(f) After the Warrant Exercise Closing Date, the Warrant Holder shall report to the Stockholder Representative in writing **[****]** and provide: **[****]**.

(g) The Stockholder Representative shall have the right, at its expense, no more than **[****]** each calendar year and not more than once with respect to the same time period and such other times as a written notice pursuant to Section 2.6(c) is given, to audit the books of the Company and, to the extent relevant, the books of the Warrant Holder, each of which the Warrant Holder shall make or cause to be made reasonably available to the Stockholder Representative, to verify the status of the Contingent Payment Events and, in the case of a written notice pursuant to Section 2.6(c) with respect to a Contingent Payment; provided that only one audit shall be conducted by the Stockholder Representative in respect of each Contingent Payment. All information obtained in any such audit shall be Confidential Information and used only for the purpose contemplated by this Section 2.6 and the Warrant Holder or the Company may require reasonable protections in the event of risk of competitive harm resulting from the disclosure. Any such audit shall be conducted upon reasonable notice during normal business hours and in a manner that does not unnecessarily or unreasonably interfere with the operations of the Company or the Warrant Holder. If, as a result of such audit, the Stockholder Representative believes that an additional Contingent Payment is due or that the amount of a Contingent Payment was understated, it shall notify the Warrant Holder in writing and shall provide the Warrant Holder reasonably detailed information reflecting the basis for the additional payment claimed. Within thirty (30) days of receipt of that notice the Warrant Holder shall either pay the additional amount claimed or notify the Stockholder Representative that the Warrant Holder disagrees with the claim, which notice shall specify the amount, if any, of the additional Contingent Payment that the Warrant Holder agrees is due. If the Parties are unable to resolve any disagreement within ten (10) days after a notice of disagreement is given by the Warrant Holder, the matter shall be referred to an independent auditor mutually agreeable to the parties, whose determination shall be final and binding upon the parties. The Warrant Holder shall make any additional Contingent Payment to the Company (and shall cause the Company to pay to the Company Equityholders in accordance with the Redemption Provisions) that the independent auditor finds to be due within ten (10) Business Days after the results of the audit are provided to the Parties. With respect to a disagreement in respect of the Sales Milestone Event, the Warrant Holder shall pay the fees, costs and expenses of any independent audit conducted to resolve such disagreement unless the independent auditor finds that the Company Equityholders are not entitled to such Contingent Payment (in which event, the Stockholder Representative shall pay such fees, costs and expenses). With respect to a disagreement in respect of the sale of a Priority Review Voucher, the percentage of the fees, costs and expenses of the audit shall be apportioned between the Warrant Holder, on the one hand, and the Stockholder Representative, on the other hand, based on the relative difference between the auditor's resolutions of disputed amounts and the relative portions of the Warrant Holder and the Stockholder Representative in respect thereof.

(h) In the event of a Change of Control of the Warrant Holder following the Warrant Exercise Closing Date with respect to which the acquiring Person or its Affiliate has a marketing approval to market and sell a product that competes with a Product (A) the Warrant Holder or entity surviving such Change of Control (or any Affiliate thereof) shall be obligated to use Commercially Reasonable Efforts to make sales of such Product, and (B) in the event that the Warrant Holder or entity surviving such Change of Control (or any Affiliate thereof) elects to use any Priority Review Voucher to accelerate the review of any product candidate to which it or

an Affiliate has rights that is not a Product Candidate, then within **[****]** days of the date of such use, the Warrant Holder shall pay a Contingent Payment to the Company and shall cause the Company to pay to the Company Equityholders pursuant to the Redemption Provisions, in an aggregate amount equal to **[****]**.

Section 2.7. Closing Date Cash and Liabilities Amount Adjustment.

(a) No more than five (5) Business Days prior to the anticipated Warrant Exercise Closing Date, and no less than two (2) days prior to the Warrant Exercise Closing Date, the Company shall in good faith prepare and deliver or cause to be prepared and delivered to the Warrant Holder the Company's estimate of the balance sheet of the Company as of 11:59 p.m. Eastern Standard Time on the day immediately prior to the Warrant Exercise Closing Date (except that Pre-Closing Tax Liabilities shall be determined as of the end of the day on the Warrant Exercise Closing Date) and deliver to the Warrant Holder a certificate signed by the Chief Financial Officer of the Company setting forth the calculation of the estimated Closing Date Cash and Liabilities Amount derived therefrom (the "Estimated Closing Date Cash and Liabilities Amount"). If the Company fails to deliver such certificate within the time period specified in this clause (a), then the Warrant Holder shall be permitted to prepare, in good faith, its estimate of the Closing Balance Sheet and the Estimated Closing Date Cash and Liabilities Amount for purposes of determining the Warrant Exercise Payment.

(b) No later than sixty (60) days after the Warrant Exercise Closing Date, the Warrant Holder shall prepare and deliver or cause to be prepared and delivered to the Stockholder Representative a balance sheet of the Company as of 11:59 p.m. Eastern Standard Time on the day immediately prior to the Warrant Exercise Closing Date (except that Pre-Closing Tax Liabilities shall be determined as of the end of the day on the Warrant Exercise Closing Date) (the "Closing Balance Sheet") and a calculation of the Closing Date Cash and Liabilities Amount. The Closing Balance Sheet shall be prepared in accordance with GAAP (except to the extent otherwise provided in the definition of Closing Date Cash and Liabilities Amount), applied in a manner consistent with the preparation, assumptions and estimates made or used in the preparation of the Financial Statements and the Estimated Closing Date Cash and Liabilities Amount; provided that to the extent the Warrant Holder incurs any prepayment penalties upon repayment of any Indebtedness included in the Estimated Closing Date Cash and Liabilities Amount prior to delivering the Closing Balance Sheet to the Company, such prepayment penalties will be included as a liability on the Closing Balance Sheet and deducted from the Closing Date Cash and Liabilities Amount. The Warrant Holder shall make available to the Stockholder Representative all records and work papers and such other information as the Stockholder Representative reasonably requests used in preparing the Closing Balance Sheet and the Closing Date Cash and Liabilities Amount. The Stockholder Representative will have a period of forty-five (45) days following the delivery of the Closing Balance Sheet to notify the Warrant Holder of any disagreements with the Closing Balance Sheet prepared by the Warrant Holder and the calculation of the Closing Date Cash and Liabilities Amount. Any such notice shall be accompanied by supporting documentation containing reasonable detail regarding such disagreement. The Stockholder Representative's failure to notify the Warrant Holder within such forty-five (45)-day period shall be deemed acceptance of the Closing Balance Sheet and the Closing Date Cash and Liabilities Amount. In the event the Stockholder Representative timely

notifies the Warrant Holder of any disagreement, the Stockholder Representative and the Warrant Holder will attempt in good faith to resolve such disagreement. If within thirty (30) days after delivery to the Warrant Holder of the notification by the Stockholder Representative of a disagreement, they are unable to resolve such disagreement, either the Warrant Holder, on the one hand, or the Stockholder Representative, on the other hand, shall have the right to submit the determination of such matter to an accounting firm of nationally recognized independent public accountants reasonably acceptable to each of the Warrant Holder and the Stockholder Representative, or in the absence of mutual agreement, an accounting firm of nationally recognized independent public accountants selected by chance after eliminating the Company's principal outside accountants prior to the Warrant Exercise Closing, the Warrant Holder's principal outside accountants and the Company's principal outside accountants (the "Auditor"). The Warrant Holder and the Stockholder Representative shall execute a reasonable engagement letter with the Auditor. Within thirty (30) days after the selection of the Auditor, the Auditor shall make a determination of all issues in dispute in connection with the Closing Balance Sheet and pursuant to such procedures as the Auditor deems fair and reasonable. The Auditor shall deliver to the Warrant Holder and the Stockholder Representative a written statement setting forth its determination of the final Closing Date Cash and Liabilities Amount and the basis for such determination. Absent manifest error, such determination shall be binding on the Parties. For the avoidance of doubt, the calculation of the Closing Date Cash and Liabilities Amount shall be made without giving effect to any of the transactions either required to occur at the Warrant Exercise Closing or otherwise occurring in connection with the Warrant Holder's consummation of the transactions contemplated by this Warrant. The percentage of the fees, costs and expenses of the Auditor shall be apportioned between the Warrant Holder, on the one hand, and the Stockholder Representative, on the other hand, based on the relative difference between the Auditor's resolutions of disputed amounts and the relative portions of the Warrant Holder and the Stockholder Representative in respect thereof.

(c) In the event that the final Closing Date Cash and Liabilities Amount (as determined in accordance with Section 2.7(b)) is greater than the Estimated Closing Date Cash and Liabilities Amount (as determined in accordance with Section 2.7(a)) (a "Closing Payment Increase"), the Warrant Holder shall pay, or shall cause to be paid, to the Company, which the Warrant Holder shall then cause the Company to pay to the Company Equityholders pursuant to the Redemption Provisions, an amount in cash equal to the Closing Payment Increase. In the event that the final Closing Date Cash and Liabilities Amount (as determined in accordance with Section 2.7(b)) is less than the Estimated Closing Date Cash and Liabilities Amount (as determined in accordance with Section 2.7(a)) (a "Closing Payment Decrease"), the Stockholder Representative shall pay to the Company from the Stockholder Representative Reserve an amount equal to the Closing Payment Decrease, which amount shall then be paid to the Warrant Holder as an adjustment to the Warrant Exercise Payment. Any payment required to be made under this Section 2.7(c) shall be made no later than five (5) Business Days after the final Closing Date Cash and Liabilities Amount is determined in accordance with Section 2.7(b).

Section 2.8. Right of Negotiation.

(a) At any time during the Review Period, the Warrant Holder may submit a written notice specifically referencing this Section 2.8(a) (an "Alternative Notice") to the Company proposing revised terms and conditions for the Warrant Exercise (the "Alternative Terms"), and the Company shall present such Alternative Terms at a meeting of the Company's Board of Directors. If **[****]**, then this Warrant shall terminate immediately, and the Company shall be free to solicit alternative Transaction Proposals from third parties. In connection with any such solicitation, the Company shall not be subject to the restrictions set forth in Section 5.12 of this Warrant to the extent necessary to provide information to any Person with respect to the rights of the Warrant Holder provided in Section 2.8(b). During the period commencing upon the date of termination of this Agreement and ending **[****]** days after the date of such termination, the Company will require that any Person interested in submitting a Transaction Proposal to the Company agree to allow disclosure of the economic and material business terms of such Transaction Proposal to the Warrant Holder and will condition its consideration of any Transaction Proposal on such agreement.

(b) If the Warrant Holder submits an Alternative Notice proposing Alternative Terms and the Warrant terminates as provided in Section 2.8(a), and, during the period commencing upon the date of such termination and ending **[****]** days after the date of such termination, the Company receives a Qualifying Acquisition Proposal or a Qualifying Licensing Proposal, then within **[****]** Business Days of such receipt by the Company, the Company shall provide written notice (the "Third Party Transaction Notice") to the Warrant Holder setting forth the economic terms of such Qualifying Acquisition Proposal or a Qualifying Licensing Proposal, along with a copy of any letter of intent or other written materials provided to the Company, any Affiliate of the Company or any of its Representatives or agents with respect to such Qualifying Acquisition Proposal or a Qualifying Licensing Proposal to the extent the Company is permitted to disclose such materials. To the extent that the Company is not permitted to disclose such materials, the Company shall provide a summary of the economic and material business terms of the underlying Transaction Proposal to the Warrant Holder. If requested by the Warrant Holder, the Company will allow an independent third party, such as a law firm, reasonably acceptable to the Warrant Holder to review such materials in order to confirm the accuracy of the summary provided by the Company. The Warrant Holder shall have the right, during the **[****]**-Business Day period following delivery of a Third Party Transaction Notice (the "Topping Period"), at its election, to submit to the Company a new transaction proposal. If the Warrant Holder submits a proposal prior to the end of the Topping Period for an alternative transaction that contains economic terms that are with respect to each individual economic term (including upfront consideration and each additional payment), at least as favorable to the Company as those contained in the Third Party Transaction Notice (a "Topping Offer"), then the Company and the Warrant Holder shall use Commercially Reasonable Efforts, during the **[****]**-day period following delivery of the Topping Offer, to finalize and enter into definitive agreements for a transaction consistent with the terms of the Topping Offer, and the Company shall negotiate exclusively with the Warrant Holder during such **[****]**-day period. For the avoidance of doubt, all material non-economic terms of this Warrant, including those in Articles 3, 4, 6 and 8, shall apply to any transaction between the Company and the Warrant Holder resulting from a Topping Offer relating to a Qualifying Acquisition Proposal. If the Warrant Holder does not submit a

Topping Offer prior to the expiration of the Topping Period or the Company and the Warrant Holder fail to execute definitive agreements with respect to a proposed transaction presented within the Topping Period within the **[****]**-day period following delivery of a Topping Offer (or such longer period as is mutually agreed between the Company and the Warrant Holder), then the Company shall be permitted, without any further obligation to the Warrant Holder under this Warrant, to (i) enter into a transaction with any third party which consists of the terms set forth in the Third Party Transaction Notice that was presented to the Warrant Holder and was the basis for the Topping Offer made by the Warrant Holder or (ii) if the **[****]**-day period following the date of the termination of this Warrant pursuant to Section 2.8(a) has expired, enter into a transaction with any third party on any terms; provided that the Company shall not, within the period ending **[****]** days after the date of the termination of this Warrant pursuant to Section 2.8(a), enter into a transaction with any third party having terms, taken as a whole, less favorable to the Company than those set forth in the Third Party Transaction Notice. For the avoidance of doubt, the Warrant Holder's rights under this Section 2.8(a) shall terminate **[****]** days after the date of the termination of this Warrant pursuant to Section 2.8(a) or, if a Third Party Transaction Notice is provided to the Warrant Holder, upon expiration of the Topping Period or, if a Topping Offer is provided by the Warrant Holder, **[****]** days following receipt by the Company of such Topping Offer.

Section 2.9. License of Intellectual Property Rights.

(a) License Grant.

(i) Effective automatically upon the receipt by the Company of the payment pursuant to Section 2.1(a) (the "License Grant Date") and subject to the terms of this Warrant, the Company hereby grants to the Warrant Holder (A) an irrevocable, exclusive, fully paid-up, perpetual, worldwide license (with the right to sublicense through multiple tiers) under any Owned Intellectual Property and Licensed Intellectual Property (in such case, owned at any time from and after the License Grant Date until the end of the Warrant Period), to develop, make, have made, use, sell, offer for sale, import, export and otherwise commercially exploit any product candidate and/or pharmaceutical product and (B) an irrevocable, non-exclusive, fully paid-up, perpetual, worldwide license (with the right to sublicense through multiple tiers) under the Company's interest in any Joint Patent to develop, make, have made, use, sell, offer for sale, import, export and otherwise commercially exploit any product candidate and/or pharmaceutical product; provided that, subject to the Company's obligations under Section 5.3, the Company shall retain rights under its Owned Intellectual Property, Licensed Intellectual Property, Joint Patents and know-how to develop, make, have made, use, sell, offer for sale, import, export and otherwise commercially exploit any product candidate and/or pharmaceutical product.

(ii) The Warrant Holder shall not exercise its rights or assume any obligations in Section 2.9(a)(i) unless (x) the Company rejects this Warrant in bankruptcy in a case under Title 11 of the United States Code, and (y) the **[****]**. All license grants under this Section 2.9(a) shall terminate immediately upon the termination of this Warrant or the Warrant Exercise Closing, unless the Company rejects this Warrant in connection with a bankruptcy proceeding.

(iii) The Company acknowledges that if the Company, as a debtor in possession or a trustee in bankruptcy in a case under Title 11 of the United States Code, rejects the Warrant or the license grant under this Section 2.9(a), the Warrant Holder may elect to retain its rights under this Section 2.9(a) as provided in Section 365(n) of Title 11 of the United States Code.

(b) Emergence from Bankruptcy.

(i) In the event that the Company emerges from bankruptcy and retains all of its material assets free and clear of any Lien and this Warrant is valid and enforceable in accordance with its terms, the Warrant Holder shall cease exercising its rights provided in Section 2.9(a)(i); provided, that if the Company subsequently rejects this Warrant again in bankruptcy in a case under Title 11 of the United States Code, the Warrant Holder shall again have the rights provided in Section 2.9(a).

Section 2.10. Withholding. Notwithstanding anything to the contrary hereunder, the Warrant Holder, the Company and any other applicable withholding agent shall be entitled to deduct and withhold, or cause to be deducted and withheld, from any portion of any payment payable pursuant to or as contemplated by this Warrant such Taxes or other amounts as it is required to deduct and withhold with respect to the making of such payment under the Code or any provision of applicable Tax Laws. To the extent that any amounts are so deducted or withheld, such amounts will be treated for all purposes of this Warrant as having been paid to the Person in respect of which such deduction and withholding was made.

ARTICLE 3
COMPANY REPRESENTATIONS AND WARRANTIES

The Company represents and warrants to the Warrant Holder that (i) except as disclosed by the Company in the Disclosure Schedule delivered on the date hereof, the following statements are true, correct and complete as of the date hereof, (ii) except as disclosed by the Company in an updated Disclosure Schedule delivered pursuant to Section 2.2 (other than pursuant to Section 2.2(c)), the following statements are true, correct and complete as of the date of delivery of such updated Disclosure Schedule and (iii) except as disclosed by the Company in the last updated Disclosure Schedule delivered pursuant to Section 2.2(c), the following statements are true, correct and complete as of the time of the Warrant Exercise. For purposes of these representations and warranties (other than those in Section 3.1, Section 3.2, Section 3.3 and Section 3.4 and Section 3.18), the term “Company” shall also include any Subsidiary of the Company, unless otherwise noted herein.

Section 3.1. Organization and Standing; Subsidiaries.

(a) The Company (a) is a corporation duly organized, validly existing and in good standing under the Laws of the jurisdiction of its incorporation, (b) has all requisite corporate power and authority required to enable it to use its corporate name and to own or lease or otherwise hold and operate its assets and properties and to carry on its business as now being conducted and (c) is duly qualified, licensed or registered to do business and is in good standing

in each of the jurisdictions listed in Section 3.1(a) of the Disclosure Schedule, which are the only jurisdictions where the nature of its business or the ownership, leasing or operation of its properties makes such qualification, licensing or registration required (except where such failure to be so qualified, licensed or registered would not reasonably be expected to result in a Material Adverse Change). The Company has made available to the Warrant Holder complete and correct copies of its Constitutive Documents, as amended. The Company has made available to the Warrant Holder copies of the stock certificate and transfer books and the minute books of the Company, each of which are true and complete in all material respects. The Company is not in violation of its Constitutive Documents.

(b) The Company does not have any Subsidiary. The Company does not own, directly or indirectly, any Capital Stock of, or other voting securities or equity interests in, any corporation, partnership, joint venture, association or other entity.

Section 3.2. Power and Authority; Binding Agreement.

(a) The Company has all requisite corporate power and authority to execute and deliver this Warrant, to issue the Warrant Shares upon the Warrant Exercise Closing and to consummate the other actions contemplated by this Warrant and the Certificate of Incorporation, including the Redemption Provisions. The execution and delivery by the Company of this Warrant, the issuance of the Warrant Shares by the Company upon the Warrant Exercise Closing and the consummation by the Company of the other actions contemplated by this Warrant and the Certificate of Incorporation, including the Redemption Provisions, have been duly authorized by all required corporate action on the part of the Company, and no other corporate proceedings on the part of the Company are required for such authorization other than the filing of a premerger notification and report form under the HSR Act and any applicable foreign competition, merger control, antitrust or similar Law, if required. This Warrant has been duly executed and delivered by the Company and, assuming due authorization, execution and delivery by the other parties thereto, constitutes a valid and binding obligation of the Company, enforceable against the Company in accordance with its terms.

(b) The execution, delivery and performance of this Warrant by the Company, the issuance of the Warrant Shares upon the Warrant Exercise Closing, and the consummation by the Company of the other actions contemplated by this Warrant do not and will not, with or without the passage of time or the giving of notice or both, (i) violate, conflict with or result in any breach of any of the terms, conditions or provisions of, or constitute a default (or give rise to any right of termination, cancellation or acceleration) under (A) the Company's Constitutive Documents, (B) any Material Contract of the Company, or (C) any provision of a Law, except where such violation, conflict or breach under (B) or (C) would not reasonably be expected to adversely affect the Company in any material respect, or (ii) result in the creation of any Lien other than a Permitted Lien, security interest, charge or encumbrance upon any of the properties or assets of the Company, except where such Lien, security interest, charge or encumbrance would not reasonably be expected to adversely affect the Company in any material respect.

(c) As of the date hereof each holder of Company Capital Stock has duly executed a consent approving, and authorizing the Company to file, the Certificate of Incorporation, including the Redemption Provisions, in substantially the form attached hereto as Exhibit C, and no holder of Company Capital Stock has revoked such approval.

Section 3.3. Authorization. The Board of Directors of the Company has duly and unanimously adopted and not later revoked, rescinded or amended resolutions (i) approving and declaring advisable this Warrant and the other transactions contemplated hereunder, (ii) duly reserving for issuance upon the Warrant Exercise Closing a sufficient number of Shares, which will, upon issuance, be validly issued, fully paid and non-assessable, (iii) determining that the payments under this Warrant are fair and declaring that this Warrant and the other transactions contemplated by this Warrant and the Certificate of Incorporation are in the best interests of the Company, and (iv) authorizing the Company to enter into this Warrant, to issue the Warrant Shares at the Warrant Exercise Closing, and to consummate the other transactions contemplated by this Warrant and the Certificate of Incorporation, on the terms and subject to the conditions set forth in this Warrant.

Section 3.4. Capitalization.

(a) Section 3.4(a) of the Disclosure Schedule sets forth the authorized capital of the Company, including the number of (x) authorized and (y) issued and outstanding shares of Company Capital Stock of each class or series.

(b) Section 3.4(b) of the Disclosure Schedule sets forth a complete and accurate list of (i) each holder of Company Capital Stock and its address in the Company stock record books, (ii) the number of shares of each class or series of Company Capital Stock held by each such stockholder and (iii) such holder's Pro Rata Percentage (as such term is defined in the Redemption Provisions). For purposes of the Disclosure Schedule delivered pursuant to Section 2.2(a) only, Section 3.4(b) of the Disclosure Schedule sets forth the Company's calculation, based on its good faith estimate of the Warrant Exercise Payment, of the aggregate payment payable to each holder of Company Capital Stock upon the Warrant Exercise Closing pursuant to the Redemption Provisions. Section 3.4(b) of the Disclosure Schedule indicates all outstanding Company Capital Stock that is Restricted Stock, indicating the name of the applicable stockholder, the vesting schedule (including any acceleration provisions with respect thereto) and the repurchase or redemption price payable by the Company. The Company holds no shares of Company Capital Stock in its treasury. All of the issued and outstanding shares of Company Capital Stock have been offered, issued and sold by the Company in material compliance with all applicable federal and state securities Laws.

(c) Section 3.4(c) of the Disclosure Schedule sets forth a complete and accurate list of (i) each holder of any securities of the Company other than Company Capital Stock and its address in the Company record books, (ii) the number of shares or principal amount of each class or series of securities of the Company held by each such holder and (iii) such holder's Pro Rata Percentage (as such term is defined in the Redemption Provisions). For purposes of the Disclosure Schedule delivered pursuant to Section 2.2(a) only, Section 3.4(c) of the Disclosure Schedule sets forth the Company's calculation, based on its good faith estimate of the Warrant Exercise Payment, of the aggregate payment payable to each holder of Company Capital Stock upon the Warrant Exercise Closing pursuant to the Redemption Provisions.

(d) Except as provided herein there are no other outstanding options, warrants, rights (including conversion rights, preemptive rights, co-sale rights, rights of first refusal or other similar rights) or agreements for the purchase or acquisition from the Company of any shares of Company Capital Stock.

(e) All of the outstanding shares of Company Capital Stock have been duly authorized and validly issued, and are fully paid and nonassessable.

(f) Section 3.4(f) of the Disclosure Schedule sets forth a complete and accurate list of (i) all holders of outstanding Company Stock Options, indicating, with respect to each Company Stock Option, the Company Stock Plan under which it was granted, the number of shares of Company Common Stock subject to such Company Stock Option, the exercise price, the date of grant, and the vesting schedule (including any acceleration provisions with respect thereto); and (ii) all holders of outstanding Company Warrants, indicating, with respect to each Company Warrant, the number of shares of Company Capital Stock, and the class or series of such shares, subject to such Company Warrant, the exercise price, the date of issuance and the expiration date thereof. Section 3.4(f) of the Disclosure Schedule sets forth a complete and accurate list of all Company Stock Plans, indicating for each Company Stock Plan the number of shares of Company Common Stock issued thereunder, the number of shares of Company Common Stock subject to outstanding options thereunder and the number of shares of Company Common Stock reserved for future issuance thereunder. There is no outstanding Company Stock Option that has not been granted under a Company Stock Plan. No Company Warrants are owned or otherwise held by any Company Personnel. The Company has made available to the Warrant Holder complete and accurate copies of all Company Stock Plans and all agreements evidencing Company Stock Options and Company Warrants. All of the shares of Company Capital Stock subject to Company Stock Options and Company Warrants will be, upon issuance pursuant to the exercise of such Contracts and Company Stock Plans, duly authorized, validly issued, fully paid and nonassessable. No Company Stock Option is exercisable for any class or series of Company Capital Stock other than Company Common Stock. Each Company Stock Option (x) was granted in material compliance with all applicable Law and all terms and conditions of the applicable Company Stock Plan and (y) has an exercise price per share of Company Common Stock equal to or greater than the fair market value of a share of Company Common Stock on the date of such grant based on a determination by the Company's Board of Directors at the time of the grant.

(g) None of the shares of Company Capital Stock (i) have been issued in violation of any subscription, Company Warrant, option, call, right of first refusal, preemptive right, conversion right, Company Stock Option, convertible security or other similar right, or any Contract to which the Company is bound or a party or (ii) are subject to any subscription, warrant, option, call, right of first refusal, preemptive right, conversion right or other similar right under any Law, the Constitutive Documents of the Company, or any Contract to which the Company is bound or a party. The Company has no obligation (contingent or otherwise), other than pursuant to the Redemption Provisions, to (A) issue or otherwise sell any subscription, warrant, option, call, right of first refusal, preemptive right, Company Stock Option, convertible security, "phantom" stock right or other similar right, or to issue, distribute or otherwise sell to holders of any shares of its Capital Stock any evidences of Indebtedness or material assets of the

Company, (B) purchase, redeem or otherwise acquire any shares of Capital Stock, or other equity or voting interest in, the Company or any other Person or to pay any dividend or to make any other distribution in respect of its Capital Stock, other than pursuant to the Redemption Provisions, or (C) vote or dispose of any shares of Capital Stock or other equity or voting interest of the Company and there are no outstanding or authorized stock appreciation rights, phantom stock awards or other similar rights that are linked in any way to the price of the Company Common Stock or the value of the Company or any part thereof. There are no equity securities of the Company reserved for issuance for any purpose, other than as disclosed on Section 3.4(g) of the Disclosure Schedule.

(h) There is no Contract between the Company and any holder of its securities, or among any holders of its securities, relating to the sale or transfer (including agreements relating to rights of first refusal, co-sale rights or "drag-along" rights), registration under the Securities Act, or voting of any Company Capital Stock.

(i) The Company does not directly or indirectly own any equity or similar interest in, or any interest convertible into or exchangeable or exercisable for any equity or similar interest in, any corporation, partnership, limited liability company, joint venture or other business association or entity. There is no Indebtedness of the Company that provides its holder with the right to vote on any matters on which stockholders of the Company may vote.

Section 3.5. Noncontravention. No consent, approval, qualification, order or authorization of, registration, declaration or filing with, or notice to, any Governmental Entity is required by or with respect to the Company in connection with the execution and delivery by the Company of this Warrant and the consummation by the Company of the transactions contemplated by this Warrant or the compliance by the Warrant Holder with the provisions of this Warrant, except for (i) the filing of a premerger notification and report form under the HSR Act, and the receipt, termination or expiration, as applicable, of approvals or waiting periods required under the HSR Act or any other applicable competition, merger control, antitrust or similar law or regulation and (ii) such other consents, approvals, orders, authorizations, registrations, declarations, filings and notices, the failure of which to be obtained or made individually or in the aggregate would not impair in any material respect the ability of the Warrant Holder to perform its obligations under this Warrant or prevent or materially impede or delay the consummation of the transactions contemplated hereunder.

Section 3.6. Compliance with Laws; Regulatory Matters.

(a) The Company is, and since its incorporation has been, in material compliance with all Laws and Judgments of any Governmental Entity applicable to its business or operations or to the conduct by the Company of its business, or the ownership or use of any of its assets and properties, including the Leased Properties. The Company has not received since the date of its incorporation a written or, to its knowledge, oral notice alleging such a possible violation by the Company of any Law or Judgment of any Governmental Entity applicable to its businesses or operations.

(b) The Company's chemical compounds and product candidates are being, and at all times have been, developed, tested, labeled, manufactured, stored, imported, exported and distributed, as applicable, in compliance in all material respects with, to the extent applicable to the Company's business, the FDCA and applicable implementing regulations issued by the FDA, the EMA and any other applicable Governmental Entities, including, as applicable, those requirements relating to the FDA's current good manufacturing practices, GLP, good clinical practices, investigational use, pre-market approval and applications to market a new pharmaceutical product and all laws referred to in EudraLex Volume 10 (Guidelines for Clinical Trials). The Company has not received written notice of, and to the knowledge of the Company the Company has not received notice of, any pending or threatened claim, suit, proceeding, hearing, enforcement, audit, investigation, arbitration or other action from the FDA alleging that any operation or activity of the Company is in violation of the FDCA or the respective counterparts thereof promulgated by applicable Governmental Entities outside the United States.

(c) The Company has made available to the Warrant Holder a complete and correct copy of all material documents and applications submitted to the FDA or EMA and all material correspondence to or from the FDA or EMA with respect to the Company's chemical compounds and product candidates, including all INDs and all supplements and amendments thereto (any such document, application, supplement or amendment, a "Regulatory Submission"), all contact reports or similar reports documenting material meetings, phone calls or other communications with the FDA or EMA that relate to substantive matters, studies, communications, memorandum and any other material written information (internal or external) required to be prepared in support of or any such material submitted in connection with each such Regulatory Submission. To the knowledge of the Company, all Regulatory Submissions (and any supporting documentation thereto) and any other written information required to be prepared in support of or any such material submitted in connection with each such Regulatory Submission, any and all requests for authorizations, approvals, certificates, waivers, certifications, clearances, notifications, licenses or permits of the FDA, EMA or any other comparable non-U.S. Governmental Entity relating to the Company, the business currently conducted by the Company and the Company's products, when submitted to the FDA or any other comparable non-U.S. Governmental Entity, including institutional review boards, independent ethics committees, or similar bodies, were true and correct in all material respects as of the date of submission.

(d) The Company has not sponsored or conducted any Clinical Trial, Exploratory IND or Exploratory CTA Study of any chemical compound or product candidate. The Company is not currently and has never in the past administered, or authorized any Person to administer on its behalf, any chemical compounds or product candidates to any human subjects, and to the knowledge of the Company, no Person has ever administered any of the Company's chemical compounds or product candidate to any human subjects, in each case other than in the course of conducting the Clinical Trials listed on Schedule 3.6(d) of the Disclosure Schedule pursuant to their respective protocols.

(e) To the extent required by applicable Legal Requirements (i) all preclinical studies and tests conducted by the Company have been, and if still pending are being, conducted in material compliance with research protocols, GLP, good clinical practices, and all applicable Legal Requirements, including, but not limited to, the FDCA and the Legal Requirements of the EMA, and, to the Company's knowledge, (ii) all preclinical studies and tests conducted on behalf of the Company have been or, if pending, are being conducted in material compliance, to the extent applicable with such practices and Legal Requirements. No preclinical study or test conducted by or, to the knowledge of the Company, on behalf of the Company has been terminated or suspended prior to completion. None of the FDA, EMA or any other applicable Governmental Entity or clinical investigator that has participated or is participating in, or institutional review board that has or has had jurisdiction over, a preclinical study or test conducted by or on behalf of the Company has commenced, or, to the knowledge of the Company, threatened to initiate, any action to place a clinical hold order on, or otherwise terminate or suspend or refuse to commence, any ongoing investigation or study conducted by or on behalf of the Company.

(f) To the extent required by applicable Legal Requirements, all animal studies or other preclinical tests performed in connection with or as the basis for any regulatory approval that has been sought or obtained for the Product Candidates, and, to the knowledge of the Company, all other animal studies or other preclinical tests performed by or on behalf of the Company, either (x) have been conducted in material compliance with all applicable Legal Requirements and rules, including, where applicable, "Good Laboratory Practice" ("GLP") as set forth in 21 CFR Part 58, the United States Animal Welfare Act, the International Conference on Harmonization's (ICH) Guideline on Nonclinical Safety Studies for the Conduct of Human Clinical Trials for Pharmaceuticals or the ICH Guideline on Safety Pharmacology Studies for Human Pharmaceuticals or (y) involved experimental research techniques that were performed for informational purposes only, whether or not included in a regulatory filing, or could not be performed by a GLP-compliant testing facility (with appropriate notice being given to the FDA in regulatory filings) and have employed the procedures and controls generally used by qualified experts in animal or preclinical studies of products comparable to those being developed by Company.

(g) The Company has not received any notice that the FDA, EMA or any other Governmental Entity, any relevant institutional review board, independent ethics committee or any other similar body has initiated, or threatened to initiate, any action to suspend any Clinical Trial conducted by or on behalf of the Company, suspend or terminate any IND sponsored by the Company or otherwise restrict or delay the preclinical or nonclinical research on or clinical study, in each case of any of the Product Candidates, or to recall, suspend or otherwise restrict the manufacture of any of the Product Candidates, or that any relevant institutional review board or independent ethics committee has refused to approve any Clinical Trial conducted by or on behalf of the Company or any substantial amendment to a protocol, any Clinical Trial conducted by or on behalf of the Company or any Exploratory IND or Exploratory CTA Study conducted by or on behalf of the Company, in each case with respect to any of the Product Candidates.

(h) The Company is not subject to any investigation that is pending and of which the Company has been notified in writing or, to the knowledge of the Company, which has been threatened, in each case by (i) the FDA or (ii) the Department of Health and Human Services Office of Inspector General or Department of Justice pursuant to the Federal Healthcare Program Anti-Kickback Statute (42 U.S.C. §1320a-7b(b)) or the Federal False Claims Act (31 U.S.C. §3729).

(i) The Company has not submitted any claim for payment to any government healthcare program in connection with any referrals related to any of the Product Candidates.

(j) The Company has not submitted any claim for payment to any government healthcare program related to any of the Product Candidates.

(k) The Company has complied in all material respects with all applicable security and privacy standards regarding protected health information under (i) the Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, including the regulations promulgated thereunder and (ii) any applicable state privacy laws.

(l) All manufacturing operations conducted by or, to the knowledge of the Company, for the benefit of the Company with respect to the Product Candidates have been and are being conducted in material compliance with applicable Legal Requirements, including, to the extent applicable, the provisions of the FDA's current good manufacturing practice regulations, and the respective counterparts thereof promulgated by the EMA and other Governmental Entities in countries outside the United States.

(m) Neither the Company nor any Company Personnel or Affiliate acting on behalf of the Company has committed any act, made any statement or failed to make any statement that would reasonably be expected to provide a basis for the FDA, the EMA or any other Governmental Entity to invoke its policy with respect to "Fraud, Untrue Statements of Material Facts, Bribery, and Illegal Gratuities" or any such similar policies set forth in any applicable Legal Requirements. None of the Company or any of its officers, directors or other Company Personnel, has been convicted of any crime or, to the knowledge of the Company, engaged in any conduct that has resulted, or would reasonably be expected to result, in debarment or exclusion under applicable Legal Requirements, including, without limitation, 21 U.S.C. Section 335a and 42 U.S.C. Section 1320a-7. No claims, actions, proceedings or investigations that would reasonably be expected to result in such a material debarment or exclusion of the Company are pending or threatened, against the Company or any of its officers, directors or, to the knowledge of the Company, other Company Personnel.

(n) There are no Legal Proceedings pending with respect to which the Company has been served and there are no other Legal Proceedings pending, in each case, with respect to an alleged violation by Company of the FDCA, regulations adopted thereunder, the Controlled Substance Act or any other Laws promulgated by the FDA, the EMA or any other Governmental Entity that applies to the regulatory status of the Company's products.

(o) The Company has not received any warning letter or untitled letter, report of inspectional observations, including FDA Form 483s, establishment inspection reports, notices of violation, clinical holds, enforcement notices or other documents from the FDA, EMA or any other similar Governmental Entity or any institutional review board or independent ethics committee alleging a lack of material compliance by Company with any Laws.

(p) The Company has not marketed, advertised, distributed, sold, or commercialized any product and is not currently marketing, distributing, selling, or otherwise commercializing any product.

Section 3.7. Permits. The Company validly holds and has in full force and effect all material Permits required by Law for it to own, lease or operate its assets and properties and to carry on its businesses as now conducted, and there exists no violation of, or default (with or without notice or lapse of time or both) under, or event giving to any Governmental Entity any right of termination, material amendment or cancellation of, any such material Permit. The Company has complied in all material respects with the terms and conditions of all Permits issued to or held by the Company, and such material Permits will not be subject to suspension, modification, revocation or nonrenewal as a result of the execution and delivery of this Warrant or the transactions contemplated hereunder. No proceeding is pending or threatened seeking the revocation or limitation of any Permit. Section 3.7 of the Disclosure Schedule lists each material Permit issued or granted to or held by the Company, true and complete copies of which have been made available to the Warrant Holder. All of the Permits listed on Section 3.7 of the Disclosure Schedule are held in the name of the Company, and none are held in the name of any Company Personnel or agent or otherwise on behalf of the Company.

Section 3.8. Financial Statements. Section 3.8 of the Disclosure Schedule sets forth (a) the consolidated balance sheet as of the most recent fiscal year end of the Company which ended at least three months prior to the date hereof or the date of any updated Disclosure Schedule submitted in accordance with Section 2.2, as applicable (the date of such balance sheet, the "Most Recent Balance Sheet Date"), and each of the preceding three (3) fiscal year ends (to the extent ending on or after December 31, 2017, if any), in each case together with the statements of income, cash flows and changes in stockholders equity for each of the fiscal years then ended, together with the footnotes thereto, and (b) the unaudited consolidated balance sheet and statements of income, changes in stockholders' equity and cash flows of the Company as of and for the period ended with the most recent month end for the month that ended at least 45 days prior to the date hereof or the date of any updated Disclosure Schedule submitted in accordance with Section 2.2, as applicable (the statements referred to in clauses (a) and (b) being referred to collectively as the "Financial Statements"). The Financial Statements (i) have been prepared from the books and records of the Company and are consistent with the books and records of the Company in all material respects, (ii) have been prepared in accordance with GAAP, consistently followed throughout the periods indicated, and (iii) present fairly, in all material respects, the consolidated financial condition, results of operations, stockholders' equity and cash flows of the Company as of the respective dates thereof and for the periods referred to therein except that the unaudited financial statements do not contain footnotes and are subject to normal year-end adjustments.

Section 3.9. Absence of Changes or Events. Between the Most Recent Balance Sheet Date and the date hereof, (a) the Company has conducted its businesses only in the Ordinary Course of Business, (b) there has occurred no Material Adverse Change, and (c) the Company has not taken any actions that, if taken after the date of this Warrant, would constitute a breach of any of the covenants set forth in Section 5.3.

Section 3.10. No Undisclosed Liabilities. The Company does not have any Liability except for (i) Liabilities reflected or reserved against in the Most Recent Balance Sheet, (ii) Liabilities that have arisen since the date of the Most Recent Balance Sheet in the Ordinary Course of Business, (iii) Liabilities arising under any Contract entered into by the Company in the Ordinary Course of Business (other than Liabilities for any breach of such Contract occurring prior to the Warrant Exercise Closing or any indemnification obligations arising under such Contract out of acts or omissions of the Company prior to the Warrant Exercise Closing), (iv) Liabilities set forth in Section 3.10 of the Disclosure Schedule and (v) except as of the Warrant Exercise Closing, Liabilities that would not be required to be shown as a liability on a balance sheet of the Company prepared in accordance with GAAP.

Section 3.11. Assets; Personal Property.

(a) The Company is the true and lawful owner and has good and valid title to all assets purported to be owned by the Company, in each case, free and clear of all Liens, other than Permitted Liens.

(b) Section 3.11(b) of the Disclosure Schedule sets forth any Contract pursuant to which the Company leases personal property as lessee or lessor with an annual payment obligation in excess of $50,000 (the "Personal Property Leases"). The Company has valid leasehold interests in all personal property purported to be leased by it pursuant to Personal Property Leases, in each case free and clear of all Liens, other than Permitted Liens. The personal property owned or leased by the Company constitutes all personal property necessary for the operation of the Company's business as presently conducted and is, in the aggregate, maintained in good operating condition, reasonable wear and tear excepted, for the purposes for which it is currently being used and is located at the offices of the Company. The Company has made available to the Warrant Holder true and correct copies of all Personal Property Leases.

Section 3.12. Real Property.

(a) The Company owns no fee title to real property.

(b) Section 3.12(b) of the Disclosure Schedule lists all interests in real property leased by the Company (each, a "Leased Property"), including the name and address of the landlord. The Company has made available to the Warrant Holder complete and accurate copies of all leases, subleases, or agreements to lease, lease guarantees, tenant estoppels, subordinations, non-disturbance, operating agreements and attornment agreements to which it is party with respect to the Leased Property. With respect to each Leased Property, (i) subject to Permitted Liens, the Company has good and valid title to the leasehold estate relating thereto, (ii) the lease relating to such Leased Property is in writing and is valid and binding, in full force

and effect and enforceable against the Company and the other parties thereto, in accordance with its terms, subject to applicable bankruptcy, insolvency, reorganization, fraudulent transfer, moratorium or similar Laws affecting creditors' rights generally, (iii) the lease relating to such Leased Property will, immediately following the date of the Warrant Exercise Closing, continue to be valid and binding, in full force and effect and enforceable against the Company and the other parties thereto, in accordance with its terms as in effect on the date hereof, subject to applicable bankruptcy, insolvency, reorganization, fraudulent transfer, moratorium or similar Laws affecting creditors' rights generally, (iv) the Company is not and to the knowledge of the Company no other party to the lease relating to such Leased Property is, in material breach or violation of, or in material default under, such lease; (v) the Company has received no written notice of any Lien, easement, covenant or other restriction applicable to such Leased Property which would reasonably be expected to materially impair the current uses or the occupancy by the Company of such Leased Property; and (vi) the Company has received no written notice that the facilities located on the Leased Property violate any zoning ordinances or similar land use requirement which would reasonably be expected to materially impair the current uses or the occupancy by the Company of such Leased Property.

Section 3.13. Contracts.

(a) Section 3.13(a) of the Disclosure Schedule lists the following Contracts that are in effect and to which the Company is a party or to which it, or any of its assets and properties, is bound and under which it has any remaining rights or obligations (each such Contract, whether or not set forth in such section of the Disclosure Schedule, a "Material Contract"):

(i) employment or consulting Contract, or any employee collective bargaining agreement or other Contract with any labor union or any Company Personnel requiring or otherwise involving payment by or to the Company of more than an aggregate of $**[****]** in the previous fiscal year;

(ii) Contract that limits or would, after the date of the Warrant Exercise Closing, limit the freedom of the Company or any of its Affiliates to compete in any line of business or geographic or therapeutic area or to make use of any of their Intellectual Property rights in any material respect;

(iii) Contract containing any material "non-solicitation" or "no-hire" provision that restricts the Company;

(iv) Contract (or substantially related Contracts) for the purchase or sale of products or the furnishing or receipt of services (1) calling for performance over a period of more than one year, (2) requiring or involving payment by or to the Company of more than an aggregate of $**[****]**, (3) in which the Company has granted exclusive manufacturing rights, "most favored nation" pricing provisions or exclusive marketing or distribution rights relating to any products or territory or (4) in which the Company has agreed to purchase a minimum quantity of goods or services or has agreed to purchase

goods or services exclusively from a certain party and is not terminable upon less than **[****]** days' notice;

(v) Contract involving the disposition or acquisition of any product line, business or significant portion of the assets, properties or business of the Company, or any merger, consolidation or similar business combination transaction, whether or not enforceable;

(vi) Contract providing for capital expenditures or other purchases of material, supplies, equipment or other assets or properties in excess of $**[****]** (other than purchase orders for inventory or supplies in the Ordinary Course of Business);

(vii) Contract for the establishment of any joint venture, equity partnership, joint product development, strategic alliance or co-marketing arrangement;

(viii) Contract granting a third party a license or sublicense to any Company Intellectual Property, or pursuant to which the Company has been granted by a third party any license or sublicense to any Intellectual Property used or planned to be used in connection with the development, manufacture or commercialization of any Product Candidate or Product or any other material Intellectual Property, except, in each case, for standard end-user, internal use software licenses for the use of commercial "shrink-wrapped" software;

(ix) any Contract to which the Company is a party as of the date hereof relating to research services or clinical trials services in respect of products (including products under development) of the Company;

(x) any right of first refusal, right of first negotiation or right of first offer in favor of a party other than the Company;

(xi) any agency, dealer, sales representative, distribution, marketing or other similar agreement;

(xii) Contract (other than trade debt incurred in the Ordinary Course of Business) under which the Company has borrowed (or may borrow) any money from, or issued (or may issue) any note, bond, debenture or other evidence of Indebtedness to, any Person;

(xiii) Contract (including so-called take-or-pay or keepwell agreements) under which (1) any Person (excluding the Company) has directly or indirectly guaranteed or assumed Indebtedness, Liabilities or obligations of the Company or (2) the Company has directly or indirectly guaranteed or assumed Indebtedness, Liabilities or obligations of any Person (in each case other than endorsements for the purpose of collection in the Ordinary Course of Business);

(xiv) Contract under which the Company has made, directly or indirectly, any advance, loan, extension of credit or capital contribution to, or other investment in, any Person (other than the Company) or any Contract providing for the making of any such advance, loan, extension of credit, capital contribution or other investment;

(xv) mortgage or other Lien, other than Permitted Liens, upon any material real property, Leased Property or other assets of the Company;

(xvi) Contract providing for indemnification of any Person (other than Contracts entered into in the Ordinary Course of Business that do not provide for uncapped indemnification obligations on the Company);

(xvii) Contract for the resolution or settlement of any actual or threatened litigation, arbitration, claim or other dispute involving payments in excess of $**[****]**;

(xviii) Contract containing any covenant not to sue, concurrent use agreement, settlement agreement, pre-rights declarations, co-existence agreement or other consent with respect to the material Company Intellectual Property;

(xix) each Company Warrant, if any; and

(xx) any other Contract involving future payments in excess of $**[****]**.

(b) Each Material Contract is valid and binding, in full force and effect and enforceable against the Company and the other parties thereto, in accordance with its terms, subject to applicable bankruptcy, insolvency, reorganization, fraudulent transfer, moratorium or similar Laws affecting creditors' rights generally. A true, correct and complete copy of each written Material Contract and a true, correct and complete summary of each oral Material Contract have been made available to the Warrant Holder. There is no material violation, breach or default under any Material Contract by the Company or, to the knowledge of the Company, by any other party thereto, and, to the knowledge of the Company, no event has occurred or condition exists that with the lapse of time or the giving of notice of any default or claimed or purported or alleged default which, with notice or the lapse of time or both, would constitute a default on the part of any party in the performance or payment of any Material Contract. No notice, waiver, consent or approval is required (or the lack of which would give rise to a right of termination, cancellation or acceleration of, or entitle any party to accelerate, whether after the giving of notice or lapse of time or both, any material obligation under the Material Contracts) under any Material Contract in connection with the execution, delivery and performance of this Warrant or the consummation of the transactions contemplated hereby.

Section 3.14. Intellectual Property.

(a) To the Company's knowledge, the Company owns, licenses, sublicenses or otherwise possesses legally enforceable rights to use all Intellectual Property required or material to the conduct of the business of the Company as and where conducted on the date hereof and as and where currently contemplated to be conducted. Section 3.14(a) of the

Disclosure Schedule sets forth a true and complete list of all Company Intellectual Property. For each item of Company Intellectual Property listed, Section 3.14(a) of the Disclosure Schedule indicates, to the extent applicable, the title, country, inventors, application number, patent number, filing date, issue date (if issued), any continuity relationship (such as continuation, continuation-in-part, divisional) with respect to any other Patent or Patent application whether such item is in effect, expires or abandoned, and, if applicable, the license agreement pursuant to which the Company receives its license under such Company Intellectual Property. Where jointly owned, Section 3.14(a) of the Disclosure Schedule indicates any other Person that has an interest in the Owned Intellectual Property, and the nature of the interest. Where licensed, the Schedule indicates the identity of the licensor and the exclusive or non-exclusive nature of such license. Complete and correct copies of all items of Company Intellectual Property have been made available by the Company to the Warrant Holder (including true and complete copies of all related license agreements, and amendments and modifications thereto).

(b) The Company owns, on an exclusive basis, free and clear of any Liens, other than Permitted Liens, or any other claims, including, without limitation, any claim of ownership or other right by any inventor on any Patent, all Owned Intellectual Property. The Company has the legal power to convey to a successor all of its ownership and license interests in the Company Intellectual Property. The Company has the right under the Company Intellectual Property to grant to the Warrant Holder the licenses set forth in Section 2.9(a) and Section 5.22(b), and it has not granted any license or other right under the Company Intellectual Property that is inconsistent with the licenses granted to the Warrant Holder hereunder. For each of the Patents listed in Section 3.14(a) of the Disclosure Schedule that is Owned Intellectual Property, no Person, other than those Persons named as inventors on such Patents, is an inventor of the invention(s) claimed by such Patent.

(c) All Owned Intellectual Property has been properly assigned to the Company, and all such assignments have been properly recorded in the United States Patent and Trademark Office ("PTO") or, if applicable, any appropriate foreign patent office. To the Company's knowledge, there are no facts that exist that to the Company's knowledge would reasonably be likely to give rise to a claim by any Person relating to the ownership, licensing, infringement, validity, enforceability or use of the Owned Intellectual Property or the exclusively licensed Licensed Intellectual Property or the research, development, manufacture, commercialization, or other use of any Product Candidate or Product.

(d) Except as set forth in Section 3.14(d) of the Disclosure Schedule, the Company does not pay or receive any royalty or other profit share payments to or from anyone with respect to any Owned Intellectual Property or Licensed Intellectual Property, nor has the Company licensed (or sublicensed, with respect to Licensed Intellectual Property) anyone to use any of the Owned Intellectual Property or Licensed Intellectual Property.

(e) Section 3.14(e) of the Disclosure Schedule sets forth a true and complete list of all Contracts to which the Company is a party or by which it is bound related to any of the Owned Intellectual Property and/or Licensed Intellectual Property (each, a "Company Intellectual Property Contract").

(f) There are no pending or contemplated claims to which the Company is party related to any Company Intellectual Property nor has the Company received written communication from any Person threatening the institution of any claim related to any Company Intellectual Property. The Company has not received written notice of and there are no ongoing interferences, oppositions, reissues, reexaminations or other proceedings involving any of the Owned Intellectual Property listed in Section 3.14(a) of the Disclosure Schedule, including ex parte and post-grant proceedings, in the PTO or in any foreign patent office or similar administrative agency.

(g) All rights of the Company in and to the Company Intellectual Property will be unaffected by the Warrant Exercise Closing and the other transactions contemplated hereunder.

(h) The Company is not subject to any Judgment with respect to, nor has it entered into or is it a party to any Contract that restricts or impairs Company's practice of any, Owned Intellectual Property or Licensed Intellectual Property. To the Company's knowledge, neither the use of Owned Intellectual Property or Licensed Intellectual Property as practiced by the Company nor the contemplated manufacture, use, sale, offer to sell or import of any product currently under clinical development by the Company (including the Product Candidates and Products), infringes or would infringe upon any Intellectual Property of any third party. To the Company's knowledge, the conduct of the business of the Company has not (in the development, manufacture or commercialization of any Product Candidate or Product), interfered with, infringed, violated or constituted a misappropriation of any Intellectual Property of any third party. The Company has not received any written or oral charge, complaint, claim, demand or notice alleging any such interference, infringement, violation or misappropriation (including any written or oral claim that the Company must license or refrain from using any Intellectual Property) and to the Company's knowledge, no such claim has been threatened.

(i) The Company has not entered into any consent, indemnification, forbearance to sue or settlement agreement with respect to any Owned Intellectual Property or Licensed Intellectual Property and no claims have been asserted against the Company in writing or been otherwise threatened in writing or orally by any Person with respect to the validity or enforceability of, or the Company's ownership of or right to use, the Company Intellectual Property and there is no basis for any such claim.

(j) To the Company's knowledge, each item of Company Intellectual Property is valid, enforceable and has been properly maintained.

(k) To the Company's knowledge, no third party has interfered with, infringed violated or misappropriated, or is currently interfering with, infringing, violating or misappropriating any rights under Owned Intellectual Property or exclusively Licensed Intellectual Property.

(l) No item of registered Company Intellectual Property has been finally judged or finally determined to be invalid or unenforceable, or has lapsed, expired or been abandoned or canceled or is the subject of cancellation or other adversarial proceeding, except as may have been allowed by the Company in the Ordinary Course of Business. The Company has timely made all filings and paid all fees required to be paid or filed in connection with the continued prosecution of the Mark or Patent applications listed in Section 3.14(a) of the Disclosure Schedule, except as may have been allowed by the Company in the Ordinary Course of Business.

(m) The Company has taken commercially reasonable precautions to maintain the confidentiality of all the Company's trade secrets and other proprietary and confidential information including Know-How. Company trade secrets and other proprietary and confidential information and materials, including chemical or biologic materials, has been maintained in confidence in accordance with the protection procedures customarily used by companies in the same industry as the Company to protect rights of like importance except with respect to any such information disclosed in the issued Company Patents or published patent applications within the Company Patents. The Company has not breached in any material respect any agreements of non-disclosure or confidentiality to which it is a party, and has not received notice of any claim or allegation of any such breach. All former and current Company Personnel who have contributed to or participated in the conception or development of any Owned Intellectual Property have executed and delivered to the Company a confidentiality agreement restricting such Person's right to disclose and use proprietary information and materials of the Company. All former and current Company Personnel either (i) have been party to a "work-for-hire" Contract with the Company, in accordance with applicable Law, that has accorded the Company the sole and exclusive ownership of all tangible and intangible property arising in the course of such Company Personnel's services on behalf of the Company or (ii) have executed appropriate instruments assigning, or agreements to assign, to the Company the sole and exclusive ownership of all Intellectual Property conceived during the course of their employment by the Company. To the knowledge of the Company, no former or current Company Personnel has any claim against the Company in connection with such Person's involvement in the conception and development of any Owned Intellectual Property and no such claim has been asserted or threatened. To the Company's knowledge, no former or current Company Personnel has any Patents issued or applications pending for any device, process, design or invention of any kind that is now used or needed by the Company in the furtherance of its current business operations, which Patents or applications have not been assigned to the Company, with such assignment duly recorded in the PTO.

Section 3.15. Litigation. There is no Legal Proceeding that is pending or, to the knowledge of the Company, threatened against the Company (or any directors, officers, agents or employees of the Company in their capacity as such), or any material assets or properties of the Company. There are no Judgments outstanding against the Company (or any directors, officers, agents or employees of the Company in their capacity as such), or any material assets or properties of the Company. Since the incorporation of the Company, there has not been any Legal Proceeding naming the Company that (a) resulted in a Judgment against or settlement by the Company (whether or not such Judgment or settlement was paid, in whole or in part, by an insurer of the Company or other third party), (b) resulted in any equitable relief or (c) relates to the Warrant Exercise Closing and the other transactions contemplated by the Warrant. There is no material Legal Proceeding pending by the Company or which the Company has considered initiating, against any other Person.

Section 3.16. Taxes. Except as set forth in Section 3.16 of the Disclosure Schedule:

(a) All Tax Returns of or with respect to the Company that are required to have been duly and timely filed have been duly and timely filed with the appropriate Taxing Authority. All such Tax Returns have been prepared in material compliance with all Tax Laws and are and were true, correct and complete in all respects. All Taxes owed by the Company (whether or not shown as due and payable on Tax Returns) have been timely paid in full.

(b) All Taxes that the Company has been required to deduct, collect or withhold in connection with amounts paid or owing to any employee, independent contractor, creditor, stockholder or other third party, have been duly and timely deducted, collected or withheld and have been duly and timely paid to the appropriate Taxing Authority, and the Company has complied with all associated reporting and record keeping requirements.

(c) No dispute, audit, investigation, proceeding or claim concerning any Liability for Taxes or Tax Returns of the Company has been raised by a Taxing Authority and no such dispute, audit, investigation, proceeding, or claim is threatened, pending, being conducted, or claimed. The Company has made available to the Warrant Holder true, correct and complete copies of all Tax Returns, examination reports, and statements of deficiencies filed, assessed against, or agreed to by the Company since the date of its incorporation.

(d) The Company has not received any written notice of deficiency, assessment or underpayment of Taxes or of adjustment of any Tax items and there is no proposed deficiency, assessment or adjustment from any federal, state, local or other Taxing Authority which could affect the Company.

(e) There are no Liens for Taxes (other than statutory liens for current Taxes not yet due and payable) on any of the assets and properties of the Company.

(f) No claim has ever been made by a Taxing Authority, in a jurisdiction where the Company does not file Tax Returns or does not pay Taxes, that the Company is (or may be) required to file Tax Returns in or be subject to Tax by that jurisdiction.

(g) No agreement or arrangement extending, or having the effect of extending, the period of assessment or collection of any Taxes payable by the Company is in effect, and the Company is not the beneficiary of any extension of time within which to file any Tax Return. There is no power of attorney given by or binding upon the Company with respect to Taxes or Tax Returns. No closing agreements, private letter rulings, technical advice memorandum or similar agreements or rulings relating to Taxes have been entered into or issued by any Taxing Authority with or in respect of the Company.

(h) The unpaid Taxes of the Company for all Pre-Closing Tax Periods as of the end of the day on the date of the Warrant Exercise Closing shall not exceed the amount of Taxes included as a liability in the calculation of Closing Date Cash and Liabilities Amount, as finally determined.

(i) The unpaid Taxes of the Company did not (i) as of the Most Recent Balance Sheet Date exceed the reserve for Taxes (excluding any reserve for Taxes established to reflect timing differences between book and Tax income) set forth on the face of the balance sheet as of such date (rather than in any notes thereto) (audited, if available), and (ii) will not, as of the date of the Warrant Exercise Closing, exceed that reserve as adjusted for the passage of time through the date of the Warrant Exercise Closing in accordance with the past practice of the Company in filing its Tax Returns. Since the Most Recent Balance Sheet Date, the Company has not incurred any liability for Taxes outside the Ordinary Course of Business.

(j) Unless approved by the Warrant Holder with respect to arrangements after the date of this Warrant, which approval will not be unreasonably withheld, the Company will not be required to include any item of income in, or exclude any item of deduction from, taxable income for federal income Tax purposes for any Post-Closing Tax Period as a result of any (i) change in method of accounting or invalid method of accounting for a Pre-Closing Tax Period, (ii) installment sale or open transaction disposition made in a Pre-Closing Tax Period, (iii) "closing agreement" as described in Code Section 7121 (or any corresponding or similar provision of state, local or foreign Tax Law) executed during a Pre-Closing Tax Period, (iv) deferred intercompany gain or excess loss account described in Treasury Regulations under Code Section 1502, (v) election under Code Section 108(i), or (vi) prepaid amount received or paid in, or deferred revenue existing in, a Pre-Closing Tax Period. There is no income of the Company that will be required under applicable domestic or foreign Tax Law to be reported by the Company or any of its Affiliates in a Post-Closing Tax Period which Taxable income was realized in (or reflects economic income arising from) a Pre-Closing Tax Period.

(k) The Company is not, and has never been within the applicable period set forth in Code Section 897(c)(1)(A)(ii), a "United States real property holding corporation" within the meaning of Code Section 897.

(l) The Company is not, and has never been, a member of an affiliated group of corporations filing a consolidated federal income Tax Return. The Company has no Liability for the Taxes of any Person under Treasury Regulations Section 1.1502-6 (or comparable provision of domestic or foreign Tax Law), as a transferee or successor, by Contract, or otherwise.

(m) The Company has not constituted a "distributing corporation" or a "controlled corporation" in a distribution qualifying or purported to qualify for nontaxable treatment (in whole or in part) under Code Section 355(a) or under analogous provisions of domestic or foreign Tax Law.

(n) The Company is not, and has never been, a "passive foreign investment company" as defined in Code Section 1297(a) or a "personal holding company" as defined in Code Section 542(a). The Company does not have an office, fixed place of business, or "permanent establishment" in, is engaged in business in, or is required to file Tax Returns or pay Taxes in, any non-U.S. country.

(o) The Company is not a party to, or otherwise bound by or subject to, any Tax sharing, allocation or indemnification or similar agreement, provision or arrangement.

(p) The Company is not a party to any joint venture, partnership or other arrangement or Contract which could be treated as a partnership for Tax purposes.

(q) The Company does not own any property of a character, the indirect transfer of which, pursuant to the transactions contemplated in this Warrant, would give rise to any Transfer Taxes.

(r) The Company has not been a party to a transaction that is a "reportable transaction" as such term is defined in Treasury Regulations Section 1.6011-4(b) or any "tax shelter" within the meaning of Code Section 6662, or any other transaction requiring analogous disclosure on the basis of "tax shelter", questionable tax avoidance or similar grounds under provisions of domestic or foreign Tax Law other than the Code.

(s) Since December 31, 2015, the Company has not made, changed or revoked any Tax election; made any change (or filed for or requested any change) in any method of Tax accounting; filed any amended Tax Return; settled or compromised any Tax liability or issue raised in connection with any Tax Return; voluntarily approached any taxing authority in respect of prior year Taxes (including through any voluntary disclosure process); consented to any claim or assessment related to any Taxes; or entered into any closing or other agreement (including an extension or waiver of any statute of limitations) with any Taxing Authority with respect to any Taxes or Tax Returns.

(t) Section 3.16(t) of the Disclosure Schedule sets forth the following information, to the knowledge of the Company, with respect to the Company as set forth on the Company's Tax returns for the most recently ended fiscal year: (i) net operating loss carryovers, alternative tax net operating loss carryovers, business credit carryforwards, credit for prior year minimum tax, and unused foreign tax credits, and (ii) the Company's good faith determination of any ownership change that has occurred prior to the date of the Warrant Exercise Closing for purposes of Section 382 of the Code.

Section 3.17. Insurance. Section 3.17 of the Disclosure Schedule contains a complete and accurate list of all policies of fire, liability, workers' compensation, title and other forms of insurance held by or otherwise maintained by the Company, and the Company has heretofore made available to the Warrant Holder a complete and accurate copy of all such policies. All such policies (or substitute policies with substantially similar terms and underwritten by insurance carriers with substantially similar or higher ratings) are valid and subsisting and in full force and effect in accordance with their terms, all premiums with respect thereto covering all periods up to and including the date of delivery of the applicable Disclosure Schedule have been paid, and no notice of cancellation or termination (or any other threatened termination) has been received by the Company with respect to any such policy. Such policies are sufficient for material compliance by the Company with (i) all requirements of applicable Law and (ii) all Contracts to which the Company is a party, and the Company has complied in all material respects with the provisions of each such policy under which it is an insured party. The Company is not in default under any of such insurance policies and there exists no event, occurrence, condition or act which, with the giving of notice or lapse of time, would become a default thereunder. The Company has not been refused any insurance or suffered the cancellation of any insurance with respect to the assets, properties or operations of the Company by any insurance carrier to which it has applied for any such insurance or with which it has carried insurance, during the last two (2) years. There are no pending or threatened claims under any insurance policy.

Section 3.18. Benefit Plans.

(a) Section 3.18(a) of the Disclosure Schedule contains a complete and correct list of all: (i) "employee benefit plans," within the meaning of Section 3(3) of ERISA, as amended, and the rules and regulations thereunder; (ii) bonus, compensation, stock option, stock purchase, Restricted Stock, equity or equity-based compensation, stock appreciation rights, phantom stock, incentive, fringe benefit, voluntary employees' beneficiary associations under Section 501(c)(9) of the Code, profit-sharing, commission, pension, retirement, deferred compensation, day or dependent care, legal services, cafeteria, medical, life insurance, dental, vision, disability, workmen's compensation or other insurance, accident, salary continuation, severance, accrued leave, vacation, sick pay, sick leave, supplemental retirement, unemployment and welfare benefit plans, programs, arrangements, commitments and/or practices (whether or not insured); and (iii) employment, consulting, services agreement, termination, change in control, retention, and severance Contracts; for active, retired or former employees, directors or officers (or under the name of Persons controlled by any of them), whether or not any such plans, programs, arrangements, commitments, contracts, agreements and/or practices (referred to in (i), (ii) or (iii) above) are in writing; that have been established, maintained or contributed to (or with respect to which an obligation to contribute has been undertaken) or with respect to which any potential Liability is borne by the Company (including, for the purpose of this Section 3.18(a), (A) any predecessors to the Company and (B) any person that for purposes of Title I and Title IV of ERISA and Section 412 of the Code would be deemed at any relevant time to be a single employer with the Company under Section 414(b), (c), (m) or (o) of the Code or Section 4001 of ERISA (an "ERISA Affiliate")), since January 1, 2006 (collectively, the "Plans").

(b) Neither the Company nor any ERISA Affiliate currently sponsors, contributes to, maintains or has any liability (whether contingent or otherwise) under (A) a Plan that is or was subject to Title IV of ERISA or Section 412 of the Code or Section 302 of ERISA, nor have any of them ever done so or (B) a "multiemployer plan" (as defined in Section 4001(a)(3) of ERISA), nor have any of them ever done so.

(c) All Plans have been administered and operated in material compliance with their terms and with all requirements of applicable Law, including ERISA and the Code. No event or omission has occurred which would cause any Plan to fail to satisfy the relevant requirements to provide tax-favored benefits under the applicable Code Section (including, without limitation, Code Sections 105 and 125). Each Plan intended to be qualified under Section 401(a) of the Code (including each related trust intended to be exempt from taxation under Section 501(a) of the Code) is so qualified and has received a current favorable IRS determination letter or is comprised of a master or prototype plan that has received a favorable opinion letter from the IRS. Since the date of each such determination letter or opinion letter, no event has occurred and no condition exists that would result in the revocation of any such determination letter or opinion letter or that would adversely affect the qualified status of any such Plan (or the tax-exempt status of any such trust). None of the Plans provide for post-employment or retiree health, life insurance and/or other welfare benefits (except as required by Section 4980B of the Code) nor have unfunded Liabilities, and the Company has no obligation to provide any such benefits to any retired or former employees or active employees following such employees' retirement or termination of service. No non-exempt prohibited transactions (within the meaning of Section 4975 of the Code or 406 of ERISA) have occurred that are reasonably expected to give rise, directly or indirectly, to material Liability to the Company.

(d) Each Plan may be amended, terminated, or otherwise modified by the Company to the greatest extent permitted by applicable Law without material Liability to the Company. The Company does not have any commitment, intention or understanding to create, modify or terminate any Plan. No event has occurred and no condition or circumstance has existed that could result in a material increase in the benefits under or the expense of maintaining any Plan as compared to the level of benefits or expense incurred for the most recently ended fiscal year of the Company.

(e) The Company has classified correctly all individuals who have performed services for them, under each Plan, ERISA and the Code and other applicable Law as common law employees, independent contractors or leased employees.

(f) Other than routine claims for benefits made in the Ordinary Course of Business, there are no pending claims, investigations or causes of action and no claims, investigations or causes of action are threatened, against any Plan or fiduciary of any such Plan by any participant, beneficiary or Governmental Entity, and there is no basis to anticipate that any such claims will be made. There are no inspections or audits pending to be resolved or implemented by any Governmental Entity to verify compliance relating to any Plan or term and condition of employment. No Plan or any fiduciary thereof has been the direct or indirect subject of an audit, investigation or examination by any Governmental Entity.

(g) The Company does not maintain or have any obligation to contribute to any "voluntary employees' beneficiary association" within the meaning of Section 501(c)(9) of the Code or other funding arrangement for the provision of welfare benefits. The Company does not maintain any Plan which is (i) a "group health plan" (as such term is defined in Section 5000(b)(1) of the Code or Section 607(1) of ERISA) that has not been administered and operated in all respects in material compliance with the applicable requirements of Part 6 of Subtitle B of Title I of ERISA and Section 4980B of the Code or (ii) a "group health plan" (as defined in 45 Code of Federal Regulations Section 160.103) that has not been administered and operated in all respects in material compliance with the applicable requirements of the Health Insurance Portability and Accountability Act of 1996 and the regulations promulgated thereunder, and the Company is not subject to any Liability, including additional contributions, fines, Taxes, penalties or loss of Tax deduction as a result of such administration and operation. No Plan which is such a group health plan is a "multiple employer welfare arrangement," within the meaning of Section 3(40) of ERISA. Each Plan that is intended to meet the requirements of Section 125 of the Code meets such requirements, and each program of benefits for which employee contributions are provided pursuant to elections under any Plan meets the requirements of the Code applicable thereto. The Company does not maintain any Plan which is an "employee welfare benefit plan" (as such term is defined in Section 3(1) of ERISA) that has provided any "disqualified benefit" (as such term is defined in Section 4976(b) of the Code) with respect to which an excise Tax could be imposed. Each Plan subject to the Patient Protection and Affordable Care Act, as modified by the Health Care and Education Reconciliation Act ("PPACA"), and related regulations and guidance, has been operated in material compliance therewith and has been timely amended, if required, to reflect the requirements of PPACA.

(h) Full payment has been timely made of all amounts which the Company is required, under applicable Law or under any Plan, applicable collective bargaining agreement or any Contract relating to any Plan to which the Company is a party, to have paid as contributions or premiums thereto as of the last day of the most recent fiscal year of such Plan ended prior to the date hereof and such contributions or premiums have been timely deposited into the appropriate trusts or accounts. All contributions and premiums have been fully deducted for income Tax purposes and no such deduction has been challenged or disallowed by any Governmental Entity and no event has occurred and no condition or circumstance has existed that could give rise to any such challenge or disallowance. The Company has either contributed or made adequate provision for reserves to meet contributions and premiums and any other Liabilities that have not been paid or satisfied because they are not yet due under the terms of any Plan, applicable Law or related Contracts. Benefits under all Plans are as represented and have not been increased subsequent to the date as of which documents have been made available to the Warrant Holder. The Company does not have any unfunded Liabilities pursuant to any Plan that is not intended to be qualified under Section 401(a) of the Code.

(i) Each Plan (including each non-qualified deferred compensation arrangement) has been maintained in material compliance with all applicable requirements of federal and state income Tax and securities Laws including (if applicable) the requirements that the offering of interests in such Plan be registered under the Securities Act of 1933 or state "Blue Sky" laws. No Plan or other Company agreement, policy or arrangement violates Section 409A of the Code. All stock options and stock appreciation rights granted by the Company after October 3, 2004, or

which vest or vested (in whole or in part) after December 31, 2004, have (or, if already terminated, had) an exercise price that was not less than the fair market value of the underlying stock as of the date such option or right was granted.

(j) Neither the execution and delivery of the Warrant, the consummation of the transactions contemplated thereby nor the conduct of the business of the Company by the Company Personnel (either alone or in combination with any other event) will (i) terminate or modify, or give a third person a right to terminate or modify, the provisions or terms of any Plan, policy, or employment contract, arrangement or commitment or (ii) result in any payment or benefits (whether of severance pay or otherwise) or any acceleration of, vesting of or increase in payments or benefits to any employee or former employee or director of, or other present or former provider of services to, the Company. No Plan provides for the payment of severance, termination, change-in-control or any similar type of payments or benefits. The Company has not made any payments, or has been a party to any contractual obligation that could result in making payments, that have resulted, or would result, separately or in the aggregate, in the payment of any "excess parachute payment" within the meaning of Section 280G of the Code or the imposition of an excise Tax under Section 4999 or that were or would not be deductible under Sections 162 or 404 of the Code, or that would be required to be included by any current or former employee, officer, director or independent contract of the Company gross income under Section 409A of the Code.

(k) The Company has made available to the Warrant Holder true and complete copies of all material documents in connection with each Plan, including (where applicable): (i) all documents embodying or governing such Plans as in effect on the date hereof, together with all amendments thereto, including, in the case of any Plan not set forth in writing, a written description thereof; (ii) all current summary Plan descriptions, summaries of material modifications, and material communications; (iii) all current trust agreements, declarations of trust and other documents establishing other funding arrangements (and all amendments thereto and the latest financial statements thereof); (iv) the most recent IRS determination letter, if any, obtained with respect to each Plan intended to be qualified under Section 401(a) of the Code or exempt under Section 501(a) or 501(c)(9) of the Code; (v) the annual report on IRS Form 5500 for each of the last two (2) years for each Plan required to file such form, with all applicable schedules and accountants' opinions attached thereto; (vi) all Contracts relating to each Plan, including service provider agreements, insurance contracts (including any fiduciary liability insurance policy or fidelity bond), annuity contracts, investment management agreements, subscription agreements, participation agreements, and recordkeeping agreements and collective bargaining agreements; (vii) the three most recent annual ADP/ACP nondiscrimination tests for any Plan intended to be qualified under Section 401(k) of the Code; (viii) any registration statement or other filing made pursuant to federal or state securities Law; (ix) all correspondence to and from any Governmental Entity with respect to the Plans; and (x) all minutes with respect to the meetings of each Plans' administrative committee or plan administrator.

Section 3.19. Employee and Labor Matters.

(a) There is no (i) strike, work slowdown, lockout, work stoppage, or picketing with respect to employees of the Company pending or threatened against or affecting the Company, and there have been no such troubles, (ii) grievance or arbitration proceeding arising out of collective bargaining agreements to which the Company is a party, including any claim for the management and administration of a collective bargaining agreement, (iii) collective labor dispute, notice of infraction, assessment, administrative labor proceeding, individual labor claim, or any other labor dispute pending, or threatened against or affecting the Company (iv) unfair labor practice complaint pending or threatened against the Company, (v) collective bargaining agreement, collective bargaining convention, or other labor union Contract to which the Company is a party, or otherwise subject, (vi) employee of the Company who is represented by a union, (vii) activity or proceeding of any labor union to organize any employees of the Company, and no demand for recognition of employees of the Company has been made by, or on behalf of, any labor union, or (viii) petition that has been filed or proceedings instituted by an employee or group of employees of the Company with any labor relations board seeking representation of a bargaining representative.

(b) The Company is and has been in compliance in all material respects with all applicable Laws regarding employment and employment practices, terms and conditions of employment, including but not limited to wages and hours, the classification of employees and independent contractors, and payment of mandatory benefits, and is not engaged in any unfair labor practice.

(c) There are no claims, investigations or causes of actions pending nor threatened against the Company with respect to any employee of the Company requesting severance, reinstatement, back salaries or pay or any other additional compensation as a direct or indirect result of a wrongful termination or termination with cause or alleged wrongful termination or termination with cause.

(d) The Company has not, since the date of its incorporation, taken any action that would constitute a “mass layoff” or “plant closing” within the meaning of the Worker Adjustment Retraining and Notification (“WARN”) Act or would otherwise trigger notice requirements or liability under any state, local or foreign plant closing notice Law. No arbitration order, court decision, governmental order, or Material Contract to which the Company is a party or is subject in any way limits or restricts the Company from relocating or closing any of the operations of the Company.

(e) Section 3.19(e) of the Disclosure Schedule contains a complete and correct list, as of the date hereof, of each employee of the Company, his or her current rate of annual base salary or current wages, bonus target for the current fiscal year of the Company, job title, employment status, work location, credited service date and date of hire. No current executive or key employee has given notice of termination of employment or otherwise disclosed plans to terminate employment with the Company.

(f) To the knowledge of the Company, no officer or director of the Company is, and no other employee of the Company is, a party to or bound by any Contract, license, or covenant of any nature (other than a Contract with the Warrant Holder or any Affiliate), or subject to any Judgment of any Governmental Entity, that may materially interfere with the use of such Person's efforts to promote the interests of the Company, conflict with the business of the Company or the Warrant Exercise Closing and the other transactions contemplated by this Warrant, or that would reasonably be expected to result in a Material Adverse Change. To the knowledge of the Company, no activity of any employee of the Company as or while an employee of the Company has caused a material violation of any employment Contract, confidentiality agreement, Patent disclosure agreement, or other Contract.

Section 3.20. Environmental Matters. In the five (5) year period prior to the date hereof, the Company has complied in all material respects with all, and is not in violation in any material respect of any, applicable Environmental Laws. The Company is in material compliance with all permits and approvals required for its operations pursuant to applicable Environmental Laws. No property (including soils, groundwater, surface water, buildings or other structures) currently owned or operated by the Company has been contaminated with any Hazardous Material. No property (including soils, groundwater, surface water, buildings or other structures) formerly owned or operated by the Company was contaminated with any Hazardous Material on or prior to such period of ownership or operation. The Company is not subject to any liability for Hazardous Material disposal or contamination on any third party property. The Company is not subject to any order, decree, injunction or other material arrangement with any Governmental Entity or any material indemnity or other material Contract with any third party relating to liability under any Environmental Law. None of the properties of the Company contains any underground storage tanks, asbestos-containing material, lead products, or polychlorinated biphenyls. The Company has not released any Hazardous Material into the environment except (i) in material compliance with Law or (ii) in an amount or concentration that would not reasonably be expected to give rise to any material liability or obligation under any Environmental Law. Copies of all environmental reports, studies, assessments, sampling data and other environmental information in the possession of the Company relating to the Company or any real property currently or formerly occupied or operated in connection with the business of the Company have been made available to the Warrant Holder. The Company has not received any written notice, demand, letter, claim or request for information from any Governmental Entity or other Person indicating that it may be in violation of or subject to liability under any Environmental Law or regarding any actual, alleged, possible or potential liability arising from or relating to the presence, generation, manufacture, production, transportation, importation, use, treatment, refinement, processing, handling, storage, discharge, Release, emission or disposal of any Hazardous Material used by the Company. No Lien or "superlien" has been placed on any site owned or operated by the Company pursuant to CERCLA or any similar state, local or federal Law. The representations and warranties contained in this Section 3.20 shall constitute the Company's sole representations and warranties with respect to environmental matters.

Section 3.21. State Takeover Statutes. The Board of Directors of the Company has unanimously approved the terms of this Warrant and the consummation of the transactions contemplated by this Warrant and such approval represents all the actions required to render inapplicable to this Warrant and to the transactions contemplated by this Warrant, the restrictions on "business combinations" set forth in Section 203 of the General Corporation Law of the State of Delaware, to the extent such restrictions would otherwise be applicable to this Warrant and the transactions contemplated by this Warrant. As of the date hereof, no other state takeover statute or similar statute or regulation applies to this Warrant or the transactions contemplated by this Warrant or Redemption Provisions.

Section 3.22. Relationships with Suppliers. Since the date of the Company's incorporation and prior to the date hereof, no supplier or licensor of the Company that is material to the Company has canceled or otherwise terminated, or provided written notice to the Company of its intent, or to the knowledge of the Company threatened, to terminate, its relationship with the Company.

Section 3.23. Transactions with Affiliates. Section 3.23 of the Disclosure Schedule describes any transaction, since the date of the Company's incorporation and prior to the date hereof, between the Company, on the one hand, and any holder of Company Capital Stock (other than the Warrant Holder or any of its Affiliates), on the other hand, other than any employment Contract, Contract not to compete with the Company, Contract to maintain the confidential information of the Company, or Contract assigning Intellectual Property rights to the Company. For purposes of avoiding confusion, the word "transaction" in the preceding sentence shall mean any "transaction" of the type described in Item 404 of Regulation S-K promulgated under the Securities Exchange Act of 1934, as amended (without regard to the amount involved in such transaction, and without regard to the Company's not being subject to such regulation). No Affiliate of the Company (other than, with respect to clause (a) of this sentence, NCH): (a) owns or has any interest in any property (real or personal, tangible or intangible), Owned Intellectual Property, Licensed Intellectual Property or Contract used in or pertaining to the business of, the Company, (b) has any claim or cause of action against the Company, or (c) owes any money to, or is owed any money by (other than, with respect to any Affiliate who is an employee of the Company, wages payable in the Ordinary Course of Business), the Company. Ownership of securities of a Person whose securities are registered under the Securities Exchange Act of 1934, as amended, of 1% or less of any class of such securities shall not be deemed to be a financial interest for purposes of this Section 3.23.

Section 3.24. Brokers. Except as disclosed in Section 3.24 of the Disclosure Schedule, the Company has no Liability to any investment banker, broker, finder, consultant or intermediary in connection with the Warrant or the transactions contemplated hereunder.

Section 3.25. Anticorruption Matters.

(a) Neither the Company nor, to the knowledge of the Company, any of the Representatives of the Company acting on its behalf has, directly or indirectly, (x) taken any action in violation of any applicable anticorruption Law, including the U.S. Foreign Corrupt Practices Act ("FCPA") (15 U.S.C. § 78 dd-1 et seq.), or (y) offered, paid, given, promised to pay or give, or authorized the payment or gift of anything of value, directly or indirectly, to any Public Official, for purposes of (A) influencing any act or decision of any Public Official in his or her official capacity; (B) inducing such Public Official to do or omit to do any act in violation of his lawful duty; (C) securing any improper advantage; or (D) inducing such Public Official to use his or her influence with a Governmental Entity, or commercial enterprise owned or controlled by any Governmental Entity (including state-owned or controlled veterinary or medical facilities), in order to assist the Company, or any Person related to the Company, in obtaining or retaining business.

(b) None of the Representatives of the Company are themselves Public Officials.

(c) There have been no false or fictitious entries made in the books or records of the Company relating to any payment that the FCPA prohibits, and the Company has not established or maintained a secret or unrecorded fund for use in making any such payments.

(d) The Company does not have knowledge of any pending issues with respect to violation of any applicable anticorruption Law, including the FCPA, relating to the Company.

(e) The Company has, or will have within ninety (90) days following the date of this Warrant, ethics policies that address and prescribe compliance with applicable anticorruption Laws, including the FCPA.

Section 3.26. Export Controls and Sanctions Matters.

(a) Neither the Company nor, to the knowledge of the Company, any of the Representatives of the Company acting on its behalf has, directly or indirectly, taken any action in material violation of any applicable export control Law, trade or economic sanctions Law, or antiboycott Law, in the United States or any other jurisdiction, including: the Arms Export Control Act (22 U.S.C.A. § 2278), the Export Administration Act (50 U.S.C. App. §§ 2401-2420), the International Traffic in Arms Regulations (22 C.F.R. 120-130), the Export Administration Regulations (15 C.F.R. 730 et seq.), the Office of Foreign Assets Control Regulations (31 C.F.R. Chapter V), the Customs Laws of the United States (19 U.S.C. § 1 et seq.), the International Emergency Economic Powers Act (50 U.S.C. § 1701-1706), the U.S. Commerce Department antiboycott regulations (15 C.F.R. 560), the U.S. Treasury Department antiboycott requirements (26 U.S.C. § 999), any other export control regulations issued by the agencies listed in Part 730 of the Export Administration Regulations, or any applicable non-U.S. Laws of a similar nature.

(b) Neither the Company nor, to the knowledge of the Company, any of the Representatives of the Company acting on its behalf, is listed on the U.S. Office of Foreign Assets Control "Specially Designated Nationals and Blocked Persons" or any other similar list.

(c) The Company has, or will have within ninety (90) days following the date of this Warrant, ethics policies that address and prescribe compliance programs appropriate to ensure compliance with the Laws identified in this Section 3.26.

ARTICLE 4
WARRANT HOLDER REPRESENTATIONS AND WARRANTIES

The Warrant Holder represents and warrants to the Company that (i) except as disclosed by the Warrant Holder in the Warrant Holder Disclosure Schedule delivered on the date hereof, the following statements are true, correct and complete as of the date hereof and (ii) except as disclosed by the Warrant Holder in an updated Warrant Holder Disclosure Schedule, the following statements are true, correct and complete as of the date of delivery of such updated Warrant Holder Disclosure Schedule.

Section 4.1. Organization and Standing. The Warrant Holder is (a) a corporation duly organized, validly existing and in good standing under the Laws of its jurisdiction of incorporation, (b) has all requisite corporate power and authority required to enable it to use its corporate or other name and to own or lease or otherwise hold and operate its assets and properties and to carry on its business as now being conducted and (c) is duly qualified, licensed or registered to do business and is in good standing in each jurisdiction in which the nature of its business or the ownership, leasing or operation of its properties makes such qualification, licensing or registration required (except where such failure to be so qualified, licensed or registered would not reasonably be expected to be materially adverse to the Warrant Holder). The Warrant Holder is not in material violation of its Constitutive Documents.

Section 4.2. Power and Authority; Binding Agreement.

(a) The Warrant Holder has all requisite corporate power and authority to execute and deliver this Warrant and to consummate the actions contemplated by this Warrant and the Certificate of Incorporation. The execution and delivery by the Warrant Holder of this Warrant and the consummation by the Warrant Holder of the other actions contemplated by this Warrant and the Certificate of Incorporation have been duly authorized by all required corporate action on the part of the Warrant Holder, and no other corporate proceedings on the part of the Warrant Holder are required other than the filing of a premerger notification and report form under the HSR Act and any applicable foreign competition, merger control, antitrust or similar Law, if required. This Warrant has been duly executed and delivered by the Warrant Holder and, assuming due authorization, execution and delivery by the other parties thereto, constitutes a valid and binding obligation of the Warrant Holder, enforceable against the Warrant Holder in accordance with its terms, subject to applicable bankruptcy, insolvency, reorganization, fraudulent transfer, moratorium or similar Laws affecting creditors' rights generally and general principles of equity.

(b) The execution, delivery and performance of this Warrant by the Company and the consummation by the Warrant Holder of the actions contemplated by this Warrant do not and will not, with or without the passage of time or the giving of notice or both, (i) violate, conflict with or result in any breach of any of the terms, conditions or provisions of, or constitute a default (or give rise to any right of termination, cancellation or acceleration) under (A) the Warrant Holder's Constitutive Documents, (B) any Contract of the Warrant Holder, or (C) any provision of Law (except where such violation, conflict or breach under (B) or (C) above would not reasonably be expected to prevent or delay or impair in any material respect the ability of the Warrant Holder to perform its obligations hereunder), (ii) violate any Law or Judgment applicable to the Warrant Holder, other than any such breaches, defaults or violations that individually or in the aggregate are not likely to impair in any material respect the ability of the Warrant Holder to perform its obligations under this Warrant, or prevent or materially impede or delay the consummation of the Warrant Exercise Closing or any of the other transaction contemplated hereunder, or (iii) result in the creation of any Lien, security interest, charge or encumbrance upon any of the properties or assets of the Warrant Holder (except where such Lien, security interest, charge or encumbrance would not reasonably be expected to result in a change materially adverse to the Warrant Holder).

(c) No consent, approval, qualification, order or authorization of, registration, declaration or filing with, or notice to, any Governmental Entity is required by or with respect to the Warrant Holder in connection with the execution and delivery by the Company of this Warrant and the consummation by the Warrant Holder of the transactions contemplated by this Warrant or the compliance by the Warrant Holder with the provisions of this Warrant, except for (i) the filing of a premerger notification and report form under the HSR Act, and the receipt, termination or expiration, as applicable, of approvals or waiting periods required under the HSR Act or any other applicable competition, merger control, antitrust or similar Law and (ii) such other consents, approvals, orders, authorizations, registrations, declarations, filings and notices, the failure of which to be obtained or made individually or in the aggregate would not impair in any material respect the ability of the Warrant Holder to perform its obligations under this Warrant or prevent or materially impede or delay the consummation of the transactions contemplated hereunder.

Section 4.3. Brokers. The Warrant Holder has not employed or entered into any Contract with any investment banker, broker, finder, consultant or intermediary in connection with the transactions contemplated by this Warrant, pursuant to which the Company could be liable for the fee or commission of such investment banker, broker, finder, consultant or intermediary, or for any similar fee or commission in connection with this Warrant or the other transactions contemplated hereunder.

Section 4.4. Accredited Investor. The Warrant Holder is an accredited investor as defined in Rule 501(a) of Regulation D promulgated under the Securities Act.

Section 4.5. Experience. The Warrant Holder has sufficient knowledge and experience in investments of the type contemplated by this Warrant that it is capable of understanding and evaluating the merits and risks of this investment.

Section 4.6. Investment. The Warrant Holder is acquiring this Warrant, the Class G Preferred Stock and the Shares issuable if the Warrant Holder elects to exercise this Warrant for its own account, for investment and not for, with a view to, or in connection with, any sale or distribution thereof within the meaning of the Securities Act.

Section 4.7. Litigation. As of the date hereof, there is no Legal Proceeding pending before any Governmental Entity, or, to the knowledge of the Warrant Holder, threatened against the Warrant Holder or any of its Affiliates or any of their respective properties, which questions or challenges the validity of this Warrant or the consummation of the transactions contemplated hereunder. There is no Judgment against the Warrant Holder or any of its Affiliates or to which the Warrant Holder or any of its Affiliates is subject that would be reasonably likely to prevent, enjoin, or materially alter or delay the Warrant Exercise Closing or any of the transactions contemplated hereunder.

Section 4.8. Sufficient Funds. The Warrant Holder has sufficient funds to enable it to make all payments required to be made by it under this Warrant (assuming Warrant Exercise Closing).

ARTICLE 5
COVENANTS

Section 5.1. Data Trigger Notification. Within **[****]** days following the Trigger Event, the Company shall deliver to the Warrant Holder a written notice confirming the occurrence of the Trigger Event and attaching, sending, or providing access to an electronic copy of the Triggering Data (a "Data Trigger Notification").

Section 5.2. Diligence During the Warrant Period. The Company's goal during the Warrant Period will be to conduct the Development Program as described in the Development Plan and Budget. During the Warrant Period, the Company shall use Commercially Reasonable Efforts to conduct and complete the Development Plan and Budget. Notwithstanding the foregoing, **[****]**.

Section 5.3. Conduct of Business.

(a) During the Warrant Period, the Company shall, except as expressly permitted by the terms of this Warrant or as approved by the Warrant Holder (such approval not to be unreasonably withheld, conditioned or delayed), (i) conduct its business in the Ordinary Course of Business, (ii) use Commercially Reasonable Efforts to keep its physical assets in good working condition, (iii) use Commercially Reasonable Efforts to preserve its rights in all material Owned Intellectual Property and material Licensed Intellectual Property, (iv) use Commercially Reasonable Efforts to maintain good working relationships with the Company's material lenders, creditors, lessors, lessees, licensors, licensees, employees, contractors, distributors, developers, vendors, clients, customers, suppliers or other Persons having a material business relationship with the Company, (v) use Commercially Reasonable Efforts to comply in all material respects with all applicable Laws and obligations under any material Contracts of the Company, and (vi) use Commercially Reasonable Efforts to ensure that no Contracts entered into by the Company contain any provisions preventing the Warrant Holder from reviewing such Contracts and any data created pursuant to such Contracts in connection with its rights under this Warrant.

(b) Without limiting the generality of Section 5.3(a), except as approved by the Warrant Holder (such approval not to be unreasonably withheld, conditioned or delayed), during the Warrant Period, the Company shall not:

(i) amend its Constitutive Documents in a manner that is materially adverse to the Warrant Holder or otherwise has any adverse effect on the terms of this Warrant (it being understood that any amendment to the Redemption Provisions will be deemed to have an adverse effect on the terms of this Warrant, and it being further understood that this Section 5.3(b)(i) does not prohibit any amendment to the Certificate of Incorporation of the Company for the sole purpose of authorizing additional shares of an existing series of preferred stock or creating a new series of preferred stock to be sold and issued in connection with a bona fide equity financing as contemplated by, and subject to, Section 5.3(b)(v)**,** as long as such amendment is not otherwise materially adverse to the Warrant Holder or otherwise has any adverse effect on the terms of this Warrant);

(ii) except as otherwise provided in Section 5.3(b)(ii) of the Disclosure Schedule, declare, set aside or pay any dividend on, or make any other distribution (whether in cash, stock or property) in respect of, any Company Capital Stock to holders of Company Capital Stock from time to time outstanding;

(iii) except as otherwise provided in Section 5.3(b)(iii) of the Disclosure Schedule, split, combine or reclassify any Company Capital Stock, or issue or authorize the issuance of any other securities in respect of, in lieu of or in substitution for shares of Company Capital Stock except in connection with a financing permitted by Section 5.3(b)(v);

(iv) purchase, redeem or otherwise acquire any shares of Company Capital Stock, or any option, warrant, call or right relating to such shares, interests or other securities (including any Company Stock Options), other than (x) in connection with the conversion or exercise of any outstanding option, warrant, call or other such right, (y) any repurchase of Company Common Stock upon a termination of the employment of any Company Personnel, pursuant to a right of repurchase in favor of the Company in any Contract to which the Company and such Company Personnel are each a party and for a per share purchase price not in excess of the fair market value of such shares of Company Common Stock at the time of any such repurchase, or (z) any acquisition of Company Common Stock upon the forfeiture of unvested Restricted Stock in accordance with its terms;

(v) issue, grant, deliver or sell, pledge, transfer or permit to be transferred, or otherwise encumber or dispose of, any shares of Company Capital Stock, or any securities convertible into, or exchangeable for, or any options, warrants, calls or rights to acquire or receive, any such shares, interests or other securities or any stock appreciation rights, phantom stock awards or other rights that are linked in any way to the price of the Company Common Stock or the value of the Company or any part thereof, other than (A) the issuance of shares of Company Common Stock or Company Preferred Stock upon the exercise of Company Stock Options or Warrants or the conversion of Company Preferred

Stock, (B) the grant of Company Stock Options with an exercise price per share at least equal to the fair market value of the Company Common Stock on the date of such issuance, and (C) the issuance of shares of Company Preferred Stock or convertible promissory notes in a bona fide equity financing to one or more investors, provided that such issuance (1) is not with the purpose of creating a "poison pill" or other anti-takeover device, (2) is not convertible into any security other than Company Common Stock or Company Preferred Stock, (3) does not have a liquidation preference that results in the aggregate liquidation preferences of all Company Preferred Stock taken as a whole exceeding the Warrant Exercise Payment, and (4) does not have any material adverse effect on the terms of this Warrant; provided that for each of clauses (A), (B) or (C), the Company shall require that any Person receiving any such shares, interests or securities pursuant to this Section 5.3(b)(v) shall execute a stockholder consent in the form attached hereto as Exhibit G if such Person has not already executed such a consent;

(vi) (A) create, incur or assume any Indebtedness, or issue or sell, or amend, modify or change any term of, any debt securities or options, warrants, calls or other rights to acquire any debt securities of the Company, (B) guarantee or endorse any Indebtedness of another Person, (C) make any loans, advances or capital contributions to, or investments in, any Person other than the Company, other than in the Ordinary Course of Business, (D) enter into any "keep well" or other Contract to maintain any financial statement condition of another Person, or (E) enter into any Contract having the economic effect of any of the foregoing;

(vii) sell, license, mortgage, transfer or otherwise encumber or subject to any Lien other than a Permitted Lien, or otherwise dispose of any properties or assets, including the Leased Properties, which are material, individually or in the aggregate, to the Development Program (excluding any sale of furniture, fixtures or equipment that does not materially impact the conduct of the Company's business) other than in the Ordinary Course of Business;

(viii) acquire (x) by merging or consolidating with, or by purchasing all or substantially all of the assets of, or by purchasing all or substantially all of the Capital Stock of, or by any other manner, any business or any other Person or any division thereof, or (y) any assets, including any interest in real property, other than in the Ordinary Course of Business, that are material, individually or in the aggregate, to the Company;

(ix) take any action (other than in the Ordinary Course of Business) that would have the effect for income Tax purposes of deferring to a Post-Closing Tax Period (or portion thereof) taxable income economically accrued in a Pre-Closing Tax Period, or accelerating a deduction from a Post-Closing Tax Period (or portion thereof) to a Pre-Closing Tax Period (other than vesting with respect to outstanding equity compensation arrangements);

(x) make, change or revoke any Tax election; make any change (or file for or request any change) in any method of Tax accounting; file any amended Tax Return; settle or compromise any Tax liability or issue raised in connection with any Tax Return; voluntarily approach any taxing authority in respect of prior year Taxes (including through any voluntary disclosure process); consent to any claim or assessment related to any Taxes; or enter into any closing or other agreement (including an extension or waiver of any statute of limitations) with any Taxing Authority with respect to any Taxes or Tax Returns;

(xi) (A) employ or retain at any time more than an aggregate of ten (10) employees, (B) hire or employ any officer or any other employee or independent contractor with compensation in excess of $**[****]** (other than employees employed by the Company as of the date of this Warrant), (C) increase compensation to any employee or independent contractor, except increases in the Ordinary Course of Business, (D) grant any severance, retention, change-in-control or other similar payments that are not discharged prior to the exercise of this Warrant or are taken into account in the Closing Date Cash and Liabilities Amount; or (E) enter into any employment agreement that cannot be terminated upon thirty (30) days' notice without liability to the Company;

(xii) enter into any lease or sublease of real property (whether as a lessor, sublessor, lessee or sublessee) or modify, amend, terminate or fail to exercise any right to renew any lease or sublease of real property, in each case other than in the Ordinary Course of Business;

(xiii) enter into any Contract (or any substantially related Contracts, taken together) that:

(A) provides for a research, license, sublicense, partnership or other collaboration with any biotechnology, pharmaceutical or similar company ("Collaboration Parties") (it being understood that Collaboration Parties shall not include NCH or any academic or research institutions and companies that primarily perform fee for service research or manufacturing services from which the Company receives only fee for service manufacturing and/or research and development services);

(B) provides for the out-license of any Company Intellectual Property to any third party, other than incidental rights granted to or retained by academic research institutions, and/or third party contractors or subcontractors that primarily perform manufacturing and/or research and development services, provided that such agreements do not grant any commercial rights to Products or Product Candidates; or

(C) involves payments to or from the Company of more than $**[****]** in any twelve (12) month period following the Warrant Exercise Closing (excluding payments following the Warrant Exercise Closing that are included in the Closing Date Cash and Liabilities Amount).

(xiv) enter into any Contract if the Warrant Exercise Closing or any of the other transactions contemplated by this Warrant or the Redemption Provisions or compliance by the Company with the provisions of this Warrant or the Redemption Provisions will conflict with, or result in any violation or breach of, or default (with or without notice or lapse of time or both) under, or give rise to a right of, or result in, termination, cancellation or acceleration of any material obligation or to a loss of a material benefit under, or result in the creation of any Lien (other than a Permitted Lien) in or upon any of the material properties or assets of the Company or the Warrant Holder or any of the Warrant Holder's Affiliates under, or give rise to any increased, additional or accelerated rights, payments or entitlements under, any provision of such Contract;

(xv) enter into any material Contract with any Affiliate of the Company, other than a Contract relating to a financing in accordance with Section 5.3(b)(v) and a Contract, other than an employment agreement, that is on arms-length or better than arm's length terms and is negotiated in good faith by the parties thereto;

(xvi) settle any claims in such a manner as would reasonably be expected to have a Material Adverse Change or subject the Company, the Warrant Holder or any Affiliate thereof to any material Liability following the Warrant Exercise Closing;

(xvii) except as required by applicable Law, adopt or enter into any collective bargaining agreement or other labor union Contract applicable to any Company Personnel;

(xviii) commence, participate or agree to commence or participate in any plan or arrangement for the complete or partial dissolution, liquidation, merger, consolidation, restructuring, recapitalization, or other reorganization of the Company (other than the Warrant Exercise Closing and the other transactions contemplated by this Warrant), including any bankruptcy, winding up, examinership, insolvency or similar proceeding in respect of the Company;

(xix) create or have any Subsidiary of the Company;

(xx) fail to (y) maintain the Company's corporate existence, due organization and good standing under the Laws of the State of Delaware other than any lapse that does not exceed 90 days and which is fully remediated within such 90 days (z) be duly qualified or licensed to do business and be in good standing in each jurisdiction in which the nature of the Company's business or the ownership, leasing or operation of its properties makes such qualification or licensing necessary, except to the extent such failure to be duly qualified, licensed or in good standing described in this clause (z) could not reasonably be expected to result in a Material Adverse Change;

(xxi) employ or enter into any Contract with any investment banker, broker, finder or advisor in connection with the Warrant Exercise Closing or the other transactions contemplated by this Warrant other than any whose fees and expenses are deducted from the Warrant Exercise Payment pursuant to the definition of the Warrant Exercise Payment;

(xxii) intentionally violate any applicable Law or intentionally fail to comply with any Judgment, which violation or failure to comply would reasonably be expected to result in a Material Adverse Change;

(xxiii) terminate any Clinical Trial before completion unless approved unanimously by the JSC or unless a regulatory authority (including, but not limited to, the FDA) or an independent, arm's length data safety monitoring committee determines such termination is necessary or the JSC unanimously determines such termination or suspension is necessary to comply with applicable Law;

(xxiv) make or engage in any public offering of any securities of the Company;

(xxv) enter into any Contract not to compete in any line of business or geographic or therapeutic area or otherwise restricting the development, manufacture, marketing, distribution or sale of products;

(xxvi) enter into any agreement with the Stockholder Representative, or permit the Stockholder Representative to enter into any agreement, which would alter, supplement or otherwise change the requirements described in the Redemption Provisions; or

(xxvii) authorize any of, or commit, or agree, whether in writing or otherwise, to take any of, the actions prohibited in this Section 5.3(b).

Section 5.4. Access.

(a) The Company shall, upon reasonable advance notice from the Warrant Holder or its Affiliates, (i) make available for inspection by the Warrant Holder or its Affiliates and their Representatives all of the Company's properties, assets, books of accounts, records (including the work papers of the Company's independent accountants), any and all data that the Company has access to related to the Development Program, and Contracts and any other materials reasonably available to the Company requested by any of them relating to the Company and its existing and prospective businesses and assets and Liabilities at such times as the Warrant Holder may reasonably request; and (ii) make available to the Warrant Holder or its Affiliates and their Representatives the officers, other senior management and Representatives of the Company for interviews, at such times as the Warrant Holder and its Representatives may reasonably request, to verify and discuss the information furnished to the Warrant Holder or its Affiliates and their Representatives and otherwise discuss the Company's existing and prospective businesses and assets and Liabilities. Any and all such inspections, interviews, and access for investigations shall be conducted during normal business hours and in a manner that does not unreasonably interfere with the conduct of the business of the Company. The foregoing shall not require the Company to allow any access that in the Company's reasonable judgment is likely to result in the waiver of any attorney-client privilege, the disclosure of any protected Intellectual Property of any third party, or the violation of any of the Company's obligations with respect to confidentiality (including under GLP, Law or Contract to which the Company is a party) nor shall the Company have any obligation to provide unblinded clinical data.

(b) Within sixty (60) days after the end of each Quarter or portion thereof occurring during the Warrant Period, the Company shall deliver to the Warrant Holder quarterly financial statements of the Company prepared in accordance with GAAP, consistently applied, except that such financial statements need not include footnotes and will be subject to customary year-end adjustments. Within one hundred twenty (120) days after each calendar year or portion thereof occurring during the Warrant Period, the Company shall deliver to the Warrant Holder annual financial statements of the Company prepared in accordance with GAAP, consistently applied. The Warrant Holder may request, and the Company shall deliver if so requested, that the annual financial statements delivered pursuant to this Section 5.4(b) be audited by an independent certified accounting firm reasonably satisfactory to the Warrant Holder, and accompanied by an audit opinion by such accounting firm, and to the extent reasonably requested by the Warrant Holder prepared in accordance with Regulation S-X promulgated by the U.S. Securities and Exchange Commission.

(c) During the Warrant Period, the Company shall promptly provide written notice to the Warrant Holder of any meetings of which the Company has knowledge with the FDA or an analogous Regulatory Entity outside of the United States in connection with a Product Candidate and shall permit the Warrant Holder or a Representative of the Warrant Holder to attend and observe any such meeting, without any right to participate, to the extent permitted by applicable Law, such agency and, if applicable, NCH.

(d) For so long as the Warrant Holder or one of its controlled Affiliates employs **[****]**, Warrant Holder shall make [****] available to the Company as is reasonably necessary for the Company to execute the Development Program, not to exceed **[****]**% of **[****]**, pursuant to the terms of an agreement regarding intellectual property ownership and compensation negotiated in good faith and reasonably acceptable to each of the Company and the Warrant Holder.

Section 5.5. Tax Matters.

(a) The Company shall timely prepare and file any Tax Return required to be filed by the Company on or before the date of the Warrant Exercise Closing (after taking all extensions into account), and timely pay any Tax reflected thereon. The Company will not take any position on such Tax Returns that is inconsistent with past practice unless otherwise required by applicable Tax Law.

(b) The Warrant Holder will prepare or cause to be prepared any Tax Return of the Company for or including a Pre-Closing Tax Period (including a Straddle Period Tax Return) required to be filed after the date of the Warrant Exercise Closing or that was required to be filed prior to the date of the Warrant Exercise Closing but was not filed on a timely basis. If such a Tax Return (x) is filed prior to the finalization of the Warrant Exercise Payment under Section 2.7, (y) is for or includes a Pre-Closing Tax Period and (z) reports that Tax is required to be paid, the Warrant Holder shall (i) deliver a copy of such Tax Return to the Stockholder Representative reasonably in advance of filing and if, within five (5) days of receipt of such copy, the Stockholder Representative notifies the Warrant Holder that it objects to any item reflected on such Tax Return which item would actually result in a downward adjustment (or

reduce an upward adjustment) to the Warrant Exercise Payment, as finally determined under Section 2.7, the Warrant Holder shall consider in good faith all changes to such item or items requested by the Stockholder Representative that are reasonably in accordance with applicable Tax Law. All Tax Returns for or that include a Pre-Closing Tax Period (and in the case of Tax Returns prepared by the Warrant Holder under Section 5.5(b), which are filed prior to the finalization of the Warrant Exercise Payment made under Section 2.7) shall be filed in accordance with applicable Tax Law and, where not unreasonable, consistent with past practice.

(c) During the Warrant Period, the Company shall not, without the consent of the Warrant Holder, effect any extraordinary transactions (other than any such transactions expressly required by applicable Law or by this Warrant) that could result in a Tax liability to the Company in a Post-Closing Tax Period in excess of Tax liability associated with the conduct of its business in the ordinary course.

(d) Unless otherwise approved or determined by the Warrant Holder in writing, the Company shall terminate all Tax allocation, indemnity or sharing Contracts to which the Company is a party, and all powers of attorney with respect to or involving the Company, prior to the Warrant Exercise Closing.

(e) The Warrant Holder, the Company and the Stockholder Representative will cooperate fully, as and to the extent reasonably requested by the other party, in connection with any Tax matters relating to the Company (including by the provision of reasonably relevant records or information).

(f) Prior to the Warrant Exercise Closing Date, the Company shall, unless the Company provides the Warrant Holder with a computation, in form reasonably satisfactory to the Warrant Holder, performed in accordance with Section 280G of the Code with sufficient details and supporting information and documentation that demonstrate that there are no "excess parachute payments" as defined in Section 280G of the Code, the Company shall, in compliance with Section 280G of the Code, (i) submit for a vote by its stockholders (the "280G Stockholder Vote"), in accordance with Section 280G of the Code and the regulations promulgated thereunder (the "280G Rules"), to receive those payments that would constitute "parachute payments" under the 280G Rules that are equal to or exceed three times such "disqualified individual base amount" (as such terms are defined under the 280G Rules), (ii) make Commercially Reasonable Efforts to secure from the "disqualified individuals" prior to such 280G Stockholder Vote a waiver of such disqualified individuals' rights to those parachute payments described above, to the extent necessary pursuant to Federal Income Tax Regulation Section 1.280G-1, Q/A-7, absent a 280G Stockholder Vote in favor of such payments, and (iii) provide to Warrant Holder drafts of the applicable documents that the Company determines are necessary to conduct the 280G Stockholder Vote within a reasonable amount of time prior to the 280G Stockholder Vote in order to allow the Warrant Holder to comment thereon.

(g) To the extent permitted by applicable Tax Law, the Tax Returns for the Pre-Closing Tax Period or Pre-Closing Straddle Period shall be prepared as follows:

(i) Any Tax deductions related to the amounts payable to the Company Equityholders pursuant to this Warrant and pursuant to the Deal Fees and other Liabilities and Indebtedness shown on the Closing Balance Sheet shall be claimed on the Tax Return for the Pre-Closing Tax Period (or Straddle Period) ending on the Warrant Exercise Closing Date.

(ii) The Company's Tax year-end for U.S. federal income tax purposes shall close as of the end of the Warrant Exercise Closing Date, and, to the extent that applicable Laws in other Tax jurisdictions so permit, the Company's Tax year shall close as of the end of the Warrant Exercise Closing Date.

(iii) An election under Revenue Procedure 2011-29 to deduct seventy percent (70%) of any Deal Fees that are success-based fees as defined in Federal Income Tax Regulation Section 1.263(a)-5(f) shall be made.

(iv) No election shall be made under Federal Income Tax Regulation 1.1502-76(b)(2) (or any similar provision of state or local Law) to ratably allocate items of the Company.

(v) Payments to the Company Equityholders shall be treated as interest to the extent required by Sections 483 and 1274 or other analogous provision of the Code or under state or local Tax Law.

(vi) Payments by the Warrant Holder to the Company (including those which are then paid by the Company to the Company Equityholders pursuant to the Redemption Provisions) pursuant to the Agreement shall be treated as subject to Section 1032 of the Code.

(vii) The Warrant Holder (and its Affiliates) shall not, and shall cause the Company not to, without the prior written consent of the Stockholders Representative (which consent shall not be unreasonably conditioned, withheld or delayed), make or cause to be made, any amended Tax Return with respect to the Company for any Pre-Closing Tax Period.

(viii) For purposes of this Warrant, in the case of any taxable period that includes (but does not end on) the date of the Warrant Exercise (a "Straddle Period"):

(A) ad valorem and other similar Taxes ("Property Taxes") of the Company for the Pre-Closing Tax Period shall be equal to the amount of such Property Taxes for the entire Straddle Period multiplied by a fraction, the numerator of which is the number of days during the Straddle Period that are in the Pre-Closing Tax Period and the denominator of which is the number of days in the Straddle Period; and

(B) the Taxes of the Company (other than Property Taxes) for the Pre-Closing Tax Period shall be computed based on an interim closing of the books as of the close of business on the date of the Warrant Exercise (and for such purpose, the Taxable period of any partnership or other pass-through entity or any "controlled foreign corporation" (within the meaning of Code Section 957) in which the Company holds a beneficial interest shall be deemed to terminate at such time).

Notwithstanding the foregoing, the Warrant Holder (and, following the Closing, the Company) shall only be subject to the above Tax Return preparation agreements in clauses (g)(i) through (g)(iv) and clause (g)(vii) (x) until the Warrant Exercise Payment has been finally determined under Section 2.7 and (y) to the extent that failure to report in accordance with such provisions would actually result in a downward adjustment (or reduce an upward adjustment) to the Warrant Exercise Payment, as finally determined under Section 2.7. Unless otherwise required by a determination of a Governmental Entity that is final and except as provided in the preceding sentence, to the extent permitted by applicable Tax Law, the Warrant Holder shall use reasonable best efforts to prepare and file all Tax Returns (and cause the Company to file all Tax returns), including timely and properly making all agreed elections, consistently with the agreements set forth in this Section 5.5(g) and neither the Warrant Holder nor the Company shall take any position (and the Warrant Holder shall not allow the Company or any of its Affiliates to take any position) on any Tax Return that is inconsistent with the agreements set forth in this Section 5.5(g) or any election made pursuant thereto.

(h) The Warrant Holder covenants that without obtaining the prior written consent of the Stockholders Representative (which consent shall not be unreasonably withheld, conditioned or delayed), it will not, and will not cause or permit the Company or any Affiliate of the Warrant Holder, to (A) take any action on the Warrant Exercise Closing Date and after the Closing other than in the Ordinary Course of Business, unless required by Law, that could give rise to any Tax Liability of the Company with respect to a Pre-Closing Tax Period, or (B) change any material Tax election with respect to, or that has retroactive effect to, any Pre-Closing Tax Period, amend any Tax Return related to a Pre-Closing Tax Period, or compromise or settle any Tax Liability, in each case (x) until the Warrant Exercise Payment has been finally determined under Section 2.7 and (y) to the extent that such action, election, amendment, compromise or settlement would actually result in a downward adjustment (or reduce an upward adjustment) to the Warrant Exercise Payment, as finally determined under Section 2.7.

Section 5.6. Insurance. The Company shall use Commercially Reasonable Efforts to keep all insurance policies set forth on Section 3.18(a) of the Disclosure Schedule, or comparable replacements therefor, in full force and effect during the Warrant Period and such that such insurance policies will be in full force and effect immediately following the Warrant Exercise Closing Date; provided, however, that any such insurance policy may be amended or modified or substituted with another insurance policy (including a change in insurance carriers), so long as the coverage and limitations provided by such amended, modified or substituted insurance policy are substantially the same as in the respective policy set forth in Section 3.18(a) of the Disclosure Schedule.

Section 5.7. Exclusivity.

(a) During the Warrant Period, the Company shall not, nor shall it authorize or permit any of its officers, directors, stockholders or Representatives or any of its Affiliates to, directly or indirectly through another Person (and it shall instruct each such Representative not to), (i) solicit, initiate or knowingly encourage any Transaction Proposal or (ii) enter into, continue or otherwise participate in any discussions or negotiations regarding, or furnish to any Person any information in connection with, any Transaction Proposal; provided that in response to any unsolicited communications from a third party with respect to a Transaction Proposal, the Company shall be permitted to inform such third party that the Company is prohibited from participating in any discussions or negotiations with respect to a Transaction Proposal. During the Warrant Period, the Company shall, and shall direct its Representatives to, (y) immediately cease and cause to be terminated all existing discussions or negotiations with any Person conducted heretofore with respect to any Transaction Proposal and (z) promptly after the date hereof request the prompt return or destruction of all confidential information previously furnished to such Person(s) within the last 12 months for the purpose of evaluating a possible Transaction Proposal.

(b) Without limiting Section 5.7(a), it is understood that any violation of the restrictions set forth in Section 5.7(a) by any Person covered by Section 5.7(a), where such Person is purporting to act on behalf of the Company, shall be deemed to be a breach of Section 5.7(a) by the Company.

(c) During the Warrant Period, if any of the Persons listed in Section 5.7(a) receives any Transaction Proposal, the Company shall, promptly after becoming aware of such Transaction Proposal, advise the Warrant Holder orally and in writing of such Transaction Proposal, the material terms and conditions of any such Transaction Proposal or inquiry (including any material changes thereto), a copy of any written materials received from such Person making the Transaction Proposal and the identity of the Person making any such Transaction Proposal or inquiry. The Company shall (i) keep the Warrant Holder informed of the status and material details of any such Transaction Proposal and (ii) provide to the Warrant Holder as soon as practicable after receipt or delivery thereof with copies of all material correspondence and other material written material sent by or provided to the Company (or its Representatives) in connection with any such Transaction Proposal.

Section 5.8. Certain IP Matters. During the Warrant Period, the Company shall pay all maintenance fees, issue fees, renewal fees, annuities and other fees required to maintain the Company Intellectual Property listed in Section 3.14(a) of the Disclosure Schedule that are material to the performance of the Development Program and due prior to thirty (30) days after the Warrant Exercise Closing Date. Without the consent of the Warrant Holder, the Company shall not allow any rights to any Owned Intellectual Property material to the performance of the Development Program to lapse or be abandoned prior to the date of the Warrant Exercise Closing. Without the consent of the Warrant Holder, the Company shall also use Commercially Reasonable Efforts (to the extent permitted under the NCH License Agreement) to prevent the licensor of any Licensed Intellectual Property material to the performance of the Development Program from allowing the Licensed Intellectual Property to lapse or be abandoned prior to the

date of the Warrant Exercise Closing. The Company shall provide the Warrant Holder with an opportunity to review and comment on all material filings to be submitted to any Regulatory Entity by or on behalf of the Company with respect to Company Intellectual Property or any of the Product Candidates (collectively, "Regulatory Filings"). The Company shall provide the Warrant Holder with final drafts of such Regulatory Filings for its review and comment and the Warrant Holder shall provide comments on such final drafts of such Regulatory Filings within ten (10) days of receipt thereof, or such other longer period of time mutually agreed to by the Parties. The Company shall implement any reasonable comments of the Warrant Holder. With respect to the NCH Licensed Intellectual Property, to the extent the Company receives from NCH any material filings to be submitted to any Regulatory Entity by NCH, the Company shall promptly provide such filings to the Warrant Holder. The Company shall use Commercially Reasonable Efforts to provide to NCH any reasonable comments received from the Warrant Holder on such filings prior to submission.

Section 5.9. No Right to Control Company During the Warrant Period. Nothing contained in this Warrant is intended to give the Warrant Holder, directly or indirectly, the right to control or direct the Company's operations during the Warrant Period. During the Warrant Period, the Company shall exercise, consistent with the terms and conditions of this Warrant, complete control and supervision over its businesses, assets and properties.

Section 5.10. Restrictive Legend. The Company agrees to affix the following legend to each certificate or other document or instrument evidencing ownership of Company Capital Stock:

> "THE SHARES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO CERTAIN MANDATORY REDEMPTION PROVISIONS AS SET FORTH IN ARTICLE V, SECTION C OF THE COMPANY'S AMENDED AND RESTATED CERTIFICATE OF INCORPORATION, AS MAY BE AMENDED FROM TIME TO TIME (A COPY OF WHICH MAY BE OBTAINED UPON WRITTEN REQUEST FROM THE COMPANY). UPON SUCH MANDATORY REDEMPTION, THE SHARES REPRESENTED BY THIS CERTIFICATE ARE ALSO SUBJECT TO THE TERMS AND PROVISIONS OF A WARRANT ISSUED BY THE COMPANY TO SAREPTA THERAPEUTICS, INC., AS MAY BE AMENDED FROM TIME TO TIME (A COPY OF WHICH MAY BE OBTAINED UPON WRITTEN REQUEST FROM THE COMPANY). BY ACCEPTING ANY INTEREST IN SUCH SHARES, THE PERSON ACCEPTING SUCH INTEREST WILL BE DEEMED TO AGREE TO AND WILL BECOME BOUND BY ALL THE PROVISIONS OF THE AGREEMENTS AND DOCUMENTS DESCRIBED IN THIS PARAGRAPH."

In lieu of the legend set forth above, the Company shall affix the following legend to each certificate evidencing ownership of Company Preferred Stock issued by the Company:

> "THE SHARES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO CERTAIN MANDATORY REDEMPTION PROVISIONS AS SET FORTH IN

ARTICLE V, SECTION C OF THE COMPANY'S AMENDED AND RESTATED CERTIFICATE OF INCORPORATION, AS MAY BE AMENDED FROM TIME TO TIME (A COPY OF WHICH MAY BE OBTAINED UPON WRITTEN REQUEST FROM THE COMPANY). BY ACCEPTING ANY INTEREST IN SUCH SHARES, THE PERSON ACCEPTING SUCH INTEREST WILL BE DEEMED TO AGREE TO AND WILL BECOME BOUND BY ALL THE PROVISIONS AS SET FORTH IN ARTICLE V, SECTION C OF THE COMPANY'S AMENDED AND RESTATED CERTIFICATE OF INCORPORATION, AS MAY BE AMENDED FROM TIME TO TIME."

Section 5.11. Warrant Holder **[****]**. The Warrant Holder shall provide the Company with [****] prior to the Warrant Exercise Closing and while this Warrant remains in effect; provided, that the Warrant Holder shall not be obligated to provide the Company with any [****] if doing so would violate any confidentiality obligations to which the Warrant Holder is bound and for which the Warrant Holder, using Commercially Reasonable Efforts, has not obtained consent from the Person or Persons to which the obligation of confidentiality is owed to provide such [****] to the Company. [****].

Section 5.12. Confidentiality.

(a) The Company and the Warrant Holder agree that, until the conclusion of the **[****]** period beginning upon the termination of this Warrant, any information provided by either the Company or the Warrant Holder to the other Party under this Warrant shall be maintained in confidence by the receiving Party and shall not be disclosed to a third party except as set forth herein without the prior written consent of the disclosing Party, except to the extent that such information (any such information, other than information subject to clauses (i) through (iv) herein, "Confidential Information"):

(i) is known by the receiving Party at the time of its receipt, and not through a prior disclosure by the disclosing Party, as documented by the receiving Party's contemporaneous business records;

(ii) is in the public domain through no breach of this Warrant by the receiving Party;

(iii) is subsequently disclosed to the receiving Party by a third party who may lawfully do so and is not to the best of the receiving Party's knowledge under an obligation of confidentiality to the disclosing Party; or

(iv) is developed by the receiving Party independently of information received from the disclosing Party, as documented by the receiving Party's contemporaneous business records.

(b) A Party may disclose the other Party's Confidential Information as required by applicable Law; provided that the Party required to make such disclosure gives the

disclosing party notice of the required disclosure and an opportunity to seek appropriate legal relief to prevent such disclosure or limit its use and further disclosure and such disclosure is limited to the extent actually required by such Law.

(c) Each Party shall use the Confidential Information of the other Party only to the extent required (i) to accomplish the purposes of this Warrant or (ii) with respect to Confidential Information related to any licenses granted in accordance with Section 5.22. Each Party will use at least the same standard of care as it uses to protect proprietary or confidential information of its own (but in no event less than reasonable care) to ensure that its employees, agents, consultants and other Representatives do not disclose or make any unauthorized use of the Confidential Information of the other party. Each Party will promptly notify the other upon discovery of any unauthorized use or disclosure of the Confidential Information of the other Party.

(d) The Parties will coordinate in advance with each other in connection with the filing of this Warrant (including redaction of certain provisions of this Warrant) with the Securities and Exchange Commission or any stock exchange or governmental agency on which securities issued by a Party or its Affiliate are traded, and each Party will use reasonable efforts to seek confidential treatment for the terms proposed to be redacted by the other Party. The Parties will use their reasonable efforts to file redacted versions with any governing bodies which are consistent with redacted versions previously filed with any other governing bodies.

Section 5.13. Restrictive Covenant. The Company agrees that its Founders (other than Bryan Barber) shall not, directly or indirectly (whether as principal, agent, independent contractor, partner or otherwise), anywhere in the world, discover, research, develop, manufacture, market, distribute or sell, commercialize, or otherwise have any interest (financial or otherwise) in any Person engaged in discovering, researching, developing, manufacturing, marketing, distributing or selling, or commercializing any therapeutic or diagnostic products that address, or are intended to address, the effects of limb-girdle muscular dystrophy which any Product Candidate is being developed to address while such Person is an employee or consultant of the Company or an Affiliate of the Company and for a period of **[****]** years following the Warrant Exercise Closing or for a period of **[****]** years following the date such Founder's employment or consulting relationship with the Company terminates for any reason. For so long as any Founder (other than Bryan Barber) is serving as an employee or consultant of the Company, the Company shall not amend such Founder's employment or consulting agreement in any manner that would terminate or limit the covenant(s) regarding noncompetition contained therein.

Section 5.14. Company Stock Options and Warrants. At the time of the Warrant Exercise Closing, the Company shall cancel and terminate any Company Stock Plan, and each Company Stock Option and Warrant outstanding immediately prior to the Warrant Exercise Closing that has not been exercised. The Company agrees that the Board of Directors of the Company (or, if appropriate, any committee administering the Company Stock Plan) shall adopt such resolutions or take such other actions (including obtaining any required consents and paying any cash or non-cash consideration) as are required to effect the transactions described in the preceding sentence; provided, that, without the consent of the Warrant Holder, which consent

shall not be unreasonably withheld, the Company shall not amend any Company Stock Plan or any Company Warrant without the prior written consent of the Warrant Holder except as otherwise provided in Section 5.3(b)(iii).

Section 5.15. Observer Rights. The Warrant Holder shall have the right to designate, upon prior approval of the Company, which approval shall not be unreasonably withheld, one individual to attend, in a non-voting observer capacity and without any right to object or otherwise participate, all meetings of the Company's Board of Directors and any committees thereof during the Warrant Period. Such board observer shall have the right to receive notice of all meetings of the Company's Board of Directors and any committees thereof, and to receive any and all drafts and executed copies of all Board of Directors resolutions, consents and other materials circulated to the Company's Board of Directors in the manner and at the same time as delivered to the Company's Board of Directors. Such board observer, or the Warrant Holder on his or her behalf (at the election of the Warrant Holder) shall be required to execute and deliver to the Company a customary confidentiality and nondisclosure agreement reasonably acceptable to the Company prior to his or her first attendance at such meetings. The Company shall have the right to prevent access of such observer to any meeting of the Board of Directors, or committee thereof, or any portion thereof, or restrict circulation of materials to such observer, if the Company, after seeking advice of counsel, deems, in its reasonable discretion, such action is necessary due to conflicts of interest or to preserve any attorney-client privileges.

Section 5.16. Commercially Reasonable Efforts. Following the delivery of an Exercise Notice and until the earlier of the Warrant Exercise Closing Date or termination of this Warrant, the Parties agree that time is of the essence with respect to each Party's covenants and obligations under this Warrant, and each Party shall use Commercially Reasonable Efforts to take, or cause to be taken, all actions and to do, or cause to be done, and to assist and cooperate with the other Party in doing, all things, in each case necessary or advisable to permit the consummation of the Warrant Exercise Closing and the other transactions contemplated by this Warrant, including the actions to be taken by the Parties as set forth in Section 2.4, obtaining any consents, authorizations, approvals, permits, licenses, or governmental authorizations, estoppel certificates and filings under any applicable Law (including any applicable filings and receiving termination or expiration of any waiting periods under the HSR Act and any applicable foreign competition, merger control, antitrust or similar Law) required to be obtained or made which may be necessary or appropriate to permit the consummation of the transactions contemplated by this Warrant. Without limiting the foregoing, and subject to Section 5.18, in the event that (x) any claim, suit, action or proceeding of the type and having any of the effects described in Section 2.4(a)(v) is pending or threatened or (y) any Legal Restraint that could reasonably be expected to result, directly or indirectly, in any of the effects described in Section 2.4(a)(v) is in effect, then the Parties shall use Commercially Reasonable Efforts to have such claim, suit, action, proceeding or Legal Restraint vacated, reversed or made to be no longer in effect.

Section 5.17. Publicity. Any initial press release regarding the issuance of this Warrant shall be prepared jointly by the Company and the Warrant Holder; provided that any such press release shall disclose only the information regarding the terms of this Warrant set forth in Exhibit H and such press release shall not disclose directly or indirectly any amounts payable by the Warrant Holder pursuant to this Warrant, unless otherwise agreed in writing by the Warrant

Holder. Thereafter, no Party shall, and each Party shall cause its Affiliates, officers, directors, employees, advisors and other Representatives not to, issue a press release or public announcement or otherwise make any public disclosure concerning the subject matter or terms of this Warrant and shall not publicly disclose directly or indirectly any amounts payable by the Warrant Holder pursuant to this Warrant without the prior written approval of the other Party, provided, however, that any Party may make any public disclosure it believes in good faith is required by applicable Law or stock market rule and in such case such Party must, prior to making such disclosure, (a) use Commercially Reasonable Efforts to advise the other Party of such disclosure (including a copy thereof) as far in advance of such disclosure as is reasonably practicable and (b) consult with the other Party with respect to the content of such disclosure. Nothing in this Section 5.17 shall prohibit the Company from disclosing information as necessary in connection with bona fide financing activities in the Ordinary Course of Business under confidentiality obligations that are substantially similar to those set forth herein. Notwithstanding anything contained in this Section 5.17, all terms of this Warrant other than those set forth in Exhibit H shall be Confidential Information governed by Section 5.12 of this Warrant.

Section 5.18. Antitrust Notification.

(a) The Parties shall, at such time as they mutually agree, but in any event no later than as promptly as practicable following delivery by Warrant Holder of the Exercise Notice, and no later than **[****]** Business Days thereafter, or on such earlier date as is requested by the Warrant Holder, each (i) file with the FTC and the DOJ the premerger notification and report form, if any, required as a result of the transactions contemplated hereby, and shall include any supplemental information requested in connection therewith, pursuant to the HSR Act and (ii) make such other filings as are necessary or advisable in other jurisdictions in order to comply with all applicable Laws relating to competition, merger control or antitrust and shall promptly provide any supplemental information requested by applicable Governmental Entities relating thereto. Any such filing, notification and report form and supplemental information shall be in substantial compliance with the requirements of the HSR Act or such other applicable Law. The Parties shall work together and shall furnish to one another such necessary information and reasonable assistance as the other may request in connection with its preparation of any filing or submission which is necessary under the HSR Act or such other applicable Law. The Parties shall keep one another apprised of the status of any communications with, and any inquiries or requests for additional information from, the FTC, the DOJ or any other applicable Governmental Entity, and shall comply promptly with any such inquiry or request.

(b) From and after such date as the filings are made pursuant to Section 5.18(a), the Parties shall use Commercially Reasonable Efforts to obtain any clearance required under the HSR Act or such other applicable Law for the transactions contemplated hereby (any such clearance, an "Antitrust Approval"). For purposes of this Section 5.18 and Section 5.16, the "Commercially Reasonable Efforts" of the Warrant Holder shall include the submission of additional information or material to any Governmental Entity requested in connection with seeking an Antitrust Approval but shall not require the Warrant Holder, its Affiliates or the Company, as the case may be, (i) to contest any Legal Proceeding by a Governmental Entity challenging or seeking a Legal Restraint under the HSR Act or any other antitrust or competition

Law; (ii) to be subject to any conditions on the ownership or operation of any material portion of its business or assets; (iii) to dispose of or hold separate more than an immaterial portion of its business or assets or the Company's business or assets as a result of the Warrant Exercise Closing or any of the other transactions contemplated by this Warrant; or (iv) to be limited in its ability to acquire or hold, or exercise full rights of ownership of, the Shares, including the right to vote such Shares.

(c) In the event that any Antitrust Approval is obtained but expires prior to date of the Warrant Exercise Closing, the Parties shall, as promptly as practicable (but in no event later than **[****]** Business Days) thereafter, make such filings as are necessary or advisable to again obtain such Antitrust Approval, in accordance with Section 5.18(a), and shall otherwise comply with Section 5.18(a) as if such expired Antitrust Approval had never been obtained.

Section 5.19. Applications for Waivers of U.S. Manufacturing Requirements. The Company shall use Commercially Reasonable Efforts to request any Person that has licensed Intellectual Property to the Company that is subject to the requirements of 35 U.S.C. § 204 to use Commercially Reasonable Efforts to cooperate and collaborate with the Warrant Holder to apply for a waiver of the United States manufacturing requirements under 35 U.S.C. § 204 in connection with any Company Intellectual Property licensed to the Company and in connection with the preparation and submission of such application, and the Warrant Holder shall have the right to review and propose changes to such application and any related materials prior to the submission thereof.

Section 5.20. Expenses. Whether or not the Warrant Exercise Closing and the other transactions contemplated by this Warrant are consummated, and except as otherwise set forth in this Warrant, each of the Parties shall bear its own fees and expenses incurred or owed in connection with the Warrant Exercise Closing and the other transactions contemplated by this Warrant, provided that any Deal Fees shall be taken into account in the Warrant Exercise Payment and the Closing Date Cash and Liabilities Amount.

Section 5.21. Indemnification of Directors and Officers.

(a) For a period of not less than **[****]** years from the Warrant Exercise Closing, the Warrant Holder shall cause the Company to cause to be maintained in effect provisions in the Certificate of Incorporation and the Company's bylaws regarding elimination of liability of directors, and indemnification and advancement of expenses to past and present directors, officers, employees and agents of the Company ("Covered Persons") that are no less advantageous to the intended beneficiaries than the corresponding provisions in effect on the date hereof (except as may be provided by Legal Requirements); provided that in the event that any claim for indemnification or advancement of expenses is asserted or made within such **[****]** year period, all rights to indemnification and advancement of expenses shall continue until such claim is disposed of or all orders, injunctions, judgments, decrees or rulings of any Governmental Entities in connection with such claim are fully satisfied.

(b) The Company shall, prior to the Warrant Exercise Closing, with any premium or portion thereof that has not been paid as of the Warrant Exercise Closing included in

the calculation of Deal Fees, purchase a "tail" insurance policy extending the reporting period for indemnification claims arising out of acts or omissions of Covered Persons prior to the Warrant Exercise Closing for a period of **[****]** years following the Warrant Exercise Closing.

(c) The rights of each Covered Person under this Section 5.21 are intended for the benefit of and shall be enforceable by such Covered Person and such Covered Person's heirs, executors or similar Representatives. The rights under this Section 5.21 shall survive the Warrant Exercise Closing and shall not be amended in a manner that is adverse to the Covered Persons without the consent of the Covered Persons affected thereby.

(d) If the Company shall (i) consolidate with or merge into any other Person and shall not be the continuing or surviving corporation or entity of such consolidation or merger, or (ii) transfer all or substantially all of its properties and assets to any Person, then, and in each such case, proper provisions shall be made so that the successors and permitted assigns shall assume all of the obligations of the Company set forth in this Section 5.21.

Section 5.22. **[****]** License.

(a) Automatically, without further action, the Warrant Holder hereby grants to the Company **[****]**.

(b) Automatically, without further action, the Company hereby grants to the Warrant Holder and its Affiliates **[****]**. To the extent any Intellectual Property is developed by or on behalf of the Warrant Holder in the exercise of the rights granted under this Section 5.22(b), then, upon termination of this Warrant, **[****]**.

(c) **[****]**.

(d) The Parties acknowledge that the licenses granted pursuant to this Section 5.22 are for the protection of the Parties and **[****]**.

Section 5.23. Stockholder Representative.

(a) The Stockholder Representative shall be appointed in accordance with the terms and conditions of the Stockholder Representative Agreement, and the Company shall promptly (and, in any event, no later than the Warrant Exercise Closing Date) provide the Warrant Holder with written notice of any such appointment, including a copy of the instrument pursuant to which the Stockholder Representative accepts such appointment. Such written notice of appointment also shall specify the amount of the Stockholder Representative Reserve. If the Company fails to designate a Stockholder Representative in accordance with the previous sentence, then the Warrant Holder may have a court of competent jurisdiction appoint a Stockholder Representative and specify the amount of the Stockholder Representative Reserve.

(b) The Warrant Holder shall be entitled to rely on the authority of the Stockholder Representative as the agent, representative and attorney-in-fact of the Company Stockholders for all purposes under this Warrant following the Warrant Exercise Closing and shall have no Liability for any such reliance. Subject to the right of the Company Stockholders

to appoint a successor Stockholder Representative pursuant to the Stockholder Representative Agreement, the Company and the may not revoke the authority of the Stockholder Representative.

Section 5.24. R&W Insurance Policy. If requested by the Warrant Holder, the Company shall use Commercially Reasonable Efforts to assist the Warrant Holder in obtaining and binding representation and warranty insurance, solely for the benefit of the Warrant Holder, relating to Losses arising from breaches of the Company's representations and warranties contained in this Warrant and on terms reasonably acceptable to the Warrant Holder (the "R&W Insurance Policy"). The Company and the Warrant Holder shall each be responsible for one half of the R&W Insurance Policy Cost.

ARTICLE 6
GOVERNANCE

Section 6.1. Joint Steering Committee. The Parties shall establish a joint steering committee ("JSC" or "Joint Steering Committee") within thirty (30) days after the date hereof that will have the responsibility for the overall coordination and oversight of the Development Plan and associated research, development and regulatory activities of the Company and to facilitate communications between the Parties and oversee, review, and manage the research, development and regulatory activities under the Development Plan. Any amendment or modification to the Development Plan must be approved by unanimous vote of the JSC.

(a) Joint Steering Committee Membership. The Company and the Warrant Holder shall each designate three (3) representatives to serve as members of the JSC by written notice to the other Party. Either Party may designate substitutes for its representatives if one (1) or more of such Party's designated representatives are unable to be present at a meeting. From time to time each Party may replace any of its representatives, in its sole discretion, effective upon written notice to the other Party. Each Party's representatives shall have appropriate technical credentials, experience, and knowledge for their specific role within the JSC (including ongoing familiarity with the Product) and shall be duly authorized under their respective company's internal governance procedures to make the decisions or carry out the activities allocated to them under this Warrant.

(b) Joint Steering Committee Chairperson. The JSC shall be co-chaired by a JSC representative of each Party. **[****]**. The role of the co-chairpersons shall include (a) scheduling meetings no less often than once per every Quarter, unless the Parties mutually agree to meet more less often; (b) convening and presiding in person or telephonically at meetings of the JSC, including ensuring that objectives for each meeting are set and achieved, (c) preparing and circulating agendas for meetings; and (d) coordinating the delivery of draft minutes to the JSC for review and final approval. The JSC co-chairpersons shall have no additional powers or rights beyond those held by the other JSC representatives.

(c) Joint Steering Committee Meetings. The JSC shall hold at least one (1) meeting every Quarter, unless the Parties mutually agree to meet more or less often, at such times during such period as it elects to do so; provided, that notwithstanding the foregoing, the

JSC shall hold an initial meeting within thirty (30) days after the date hereof. Meetings of the JSC shall be effective only if at least one (1) representative of each Party is present or participating. The JSC may meet either (a) in person at either Party's facilities or at such locations as the Parties may otherwise agree or (b) by audio or video teleconference; provided, that no less than one (1) meeting of the JSC during each calendar year shall be conducted in person. Each Party may invite additional representatives or consultants to attend JSC meetings from time to time. Each Party shall be responsible for all of its own expenses incurred in connection with participating in the JSC meetings.

Section 6.2. Decisions of the JSC.

(a) Voting; Consensus. Subject to the remainder of this Section 6.2, the JSC will act by unanimous agreement. The representatives from each Party have, **[****]** on behalf of that Party. Except as otherwise expressly set forth in this Warrant, the phrase "approve," "determine," or "determine whether to approve" by the JSC and similar phrases used in this Warrant shall mean approval by the JSC in accordance with this Section 6.2, including the escalation and tie-breaking provisions herein.

(b) Escalation to EOs. Any disagreement between the representatives of the Parties with respect to matters properly coming before the JSC that cannot be resolved after good faith efforts will, at the election of either Party, be submitted to the JSC for resolution, If the JSC fails to reach unanimous agreement on such matter within a period of **[****]**, then either Party may immediately refer the matter for resolution to (i) the **[****]** of the Company and (ii) the [****] of the Warrant Holder, or their respective designee (such officer or such designee, the "Executive Officer"). In the event that the Executive Officers are unable to resolve such dispute within **[****]** of such dispute being referred to the Executive Officers, then the provisions of Section 6.2(c) shall apply.

(c) Subsequent Dispute Resolution Procedures. To the extent a failure of the JSC to reach unanimous agreement has not been resolved pursuant to Section 6.2(b), **[****]**.

Section 6.3. Authority. The JSC shall have only the powers assigned expressly to it in this Article 6 and elsewhere in this Warrant, and shall not have any power to amend, modify, or waive compliance with this Warrant. In furtherance thereof, each Party shall retain the rights, powers, and discretion granted to it under this Warrant and no such rights, powers, or discretion shall be delegated or vested in the JSC unless such delegation or vesting of rights is expressly provided for in this Warrant or the Parties expressly so agree in writing. Notwithstanding anything herein to the contrary, the Company shall have the right to make operational decisions that do not conflict with, or require an amendment to, the then-current Development Plan and Budget without seeking approval from the JSC.

ARTICLE 7
INDEMNIFICATION; LIMITATION OF REMEDIES

Section 7.1. Indemnification by the Company Equityholders. From and after the Warrant Exercise Closing, the Company Equityholders shall, severally in accordance with their respective Pro Rata Percentages (or in the case of clauses (c) and (d) below, severally and solely

as to itself) indemnify and hold the Warrant Holder and its Affiliates (including the Company) and the Representatives, Affiliates, successors and assigns of each of the foregoing Persons (each, a "Warrant Holder Indemnified Party") harmless from, against and in respect of any and all Losses suffered or incurred by such Warrant Holder Indemnified Parties or any of them as a result of, arising out of or directly or indirectly relating to:

(a) any Fraud on the part of the Company contained in any representation or warranty made by or on behalf of the Company contained in this Warrant or in any Disclosure Schedule or certificate delivered pursuant hereto;

(b) any breach of or failure to perform any covenant or agreement of the Company contained in this Warrant, whether occurring before or at the Warrant Exercise Closing but not after the Warrant Exercise Closing (other than as provided in Section 5.13);

(c) any Fraud on the part of such Company Equityholder contained in or incorporated by reference into the Stockholder Acknowledgment; or

(d) any breach or failure to perform any covenant or agreement of such Company Equityholder (including under this Article 7) in or incorporated by reference into the Stockholder Acknowledgment; provided, however, that no Warrant Holder Indemnified Parties shall be entitled to be indemnified for any Losses under clauses (a) through (d) above (1) unless (A) the claim for each such Loss which the Warrant Holder Indemnified Parties would, but for this proviso, incur, exceeds, $**[****]** (the "Minimum"), and (B) except for those Losses **[****]**, the aggregate of all such Losses which the Warrant Holder Indemnified Parties would, but for this proviso, incur, exceeds, on a cumulative basis, an amount equal to $**[****]** (the "Threshold"), at which point, in each case, the Warrant Holder Indemnified Parties shall be entitled to be indemnified for the aggregate of such indemnifiable Losses, and not just amounts in excess of the Minimum or Threshold, as applicable, and (2) (A) **[****]**, (B) **[****]** and (C) **[****]**. The Warrant Holder Indemnified Parties shall be entitled to the indemnification provided for hereunder even if any of them had knowledge at any time of the matter that is later the subject of a claim for indemnity.

Section 7.2. Indemnification by the Warrant Holder. From and after the Warrant Exercise Closing, the Warrant Holder shall, indemnify and hold the Company Equityholders and their respective Affiliates and the Representatives, Affiliates, successors and assigns of each of the foregoing Persons (each, a "Company Equityholder Indemnified Party") harmless from, against and in respect of any and all Losses suffered or incurred by such Warrant Holder Indemnified Parties or any of them as a result of, arising out of or directly or indirectly relating to:

(a) any breach of, or inaccuracy in, any representation or warranty made by the Warrant Holder in Article 4 of this Warrant; or

(b) any breach of or failure to perform any covenant or agreement of the Company contained in this Warrant, whether occurring before or after the Warrant Exercise Closing Date.

The Company Equityholders shall be entitled to the indemnification provided for hereunder even if they had knowledge at any time of the matter that is later the subject of a claim for indemnity. The Parties acknowledge and agree that the Stockholder Representative shall have the exclusive right to enforce this Section 7.2 on behalf of the Company Equityholders.

Section 7.3. Indemnification Claims.

(a) In order for a Party seeking indemnification under this Article 7 (an "Indemnified Party") to be entitled to any indemnification from the other Party hereunder (an "Indemnifying Party") in respect of, arising out of or involving a Third Party Claim, the Indemnified Party must notify the Indemnifying Party in writing of the Third Party Claim promptly; provided, however, that a delay of, or failure to give, such notification shall not affect the indemnification provided under Section 7.1 or Section 7.2 except to the extent the Indemnifying Party has been actually and materially prejudiced as a result of such delay or failure. Thereafter, the Indemnified Party shall deliver to the Indemnifying Party (to the extent such Indemnified Party is a Warrant Holder Indemnified Party, any notices required by this Section 7.3 shall be delivered to the Stockholder Representative), within five (5) Business Days after the Indemnified Party's receipt thereof, copies of all notices and documents (including court papers) received by the Indemnified Party relating to the Third Party Claim. The Indemnifying Party shall have the right to assume and control the defense of such Third Party Claim with counsel of its choosing reasonably acceptable to the Indemnified Party so long as (i) the Indemnifying Party acknowledges to the Indemnified Party that such Third Party Claim involves an indemnifiable matter under this Article 7, (ii) the Third Party Claim does not seek an injunction or other equitable relief against the Indemnified Party, (iii) the Indemnifying Party conducts the defense of the Third Party Claim actively and diligently, (iv) the Indemnifying Party would reasonably be expected to be financially responsible for greater than 50% of an adverse outcome and (v) the Third Party Claim does not relate to or otherwise arise in connection with Intellectual Property or any Legal Proceeding involving Tax, criminal or regulatory enforcement. The Indemnified Party shall cooperate fully with the Indemnifying Party and may participate in the defense of such Third Party Claim at its own expense. The Indemnifying Party shall keep the Indemnified Party advised of the status of such claim and the defense thereof and will not consent to the entry of any judgement or enter into any settlement with respect to the Third Party Claim without the written consent of the Indemnified Party, unless such judgement or settlement (i) includes an unconditional written release by the claimant or plaintiff of all of the Indemnified Parties from all Liability in respect of such Third Party Claim, (ii) does not involve any admission of liability or wrongdoing by any Indemnified Party or its Affiliates and (iii) does not impose equitable remedies or non diminimus obligations on the Indemnified Party other than financial obligations for which such Indemnified Party will be indemnified hereunder. No Third Party Claim which is being defended in good faith by the Indemnifying Party in accordance with the terms of this Warrant shall be settled by the Indemnified Party without the written consent of the Indemnifying Party.

(b) With respect to any claim for indemnification pursuant to Section 7.1, references in this Warrant to the "Indemnifying Party" with respect to any right to give or receive notice or consent shall be deemed to refer to the Stockholder Representative.

Section 7.4. Adjustments to Indemnification Payments. All indemnification payments under Section 7.1 and Section 7.2 shall be reduced by and paid net of any insurance coverage actually received by the Warrant Holder Indemnified Party net of any costs and expenses incurred by the Warrant Holder Indemnified Party in connection with collecting such insurance proceeds, and the Indemnified Party shall use commercially reasonable efforts to claim and recover any Losses suffered by it under any available insurance policies.

Section 7.5. Survival and Expiration of the Representations and Warranties. All representations and warranties made by or on behalf of the Parties contained in this Warrant shall expire on the Warrant Exercise Closing Date, except to the extent required to survive for any R&W Insurance Policy. Any claim for indemnification made under Section 7.1 must be raised in a writing delivered to the Indemnifying Party by no later than the date that is **[****]** after the Warrant Exercise Closing Date and, if raised by such date, such claim shall survive such date until final resolution thereof.

Section 7.6. No Right of Contribution. The Stockholder Representative shall not have any right of contribution against the Company or the Warrant Holder with respect to any breach by the Company of any of its representations, warranties, covenants or agreements.

Section 7.7. Set-Off. Upon notice to the Stockholder Representative specifying in reasonable detail the basis therefor, the Warrant Holder may set off any amount of Losses to which it is entitled under this Article 7 (taking into account the limitations on indemnification set forth in this Article 7) against Contingent Payments payable to the Company pursuant to Section 2.6. If the Warrant Holder has asserted a claim for indemnification pursuant to this Article 7, but the Warrant Holder's right to be indemnified to the extent of its claim has not yet been determined, then the Warrant Holder may withhold the amount of the claimed indemnifiable Losses (subject to the limitations set forth herein, if applicable) from a future payment to be made pursuant to Section 2.6 until the Warrant Holder's entitlement to indemnification in respect of such claim has been finally determined, at which point the Warrant Holder shall be entitled to retain the amount to which it is entitled to be indemnified hereunder, if any, and shall pay over the remainder, if any, to the Company.

Section 7.8. Exclusive Remedy. Except for any rights the Warrant Holder may have pursuant to the R&W Insurance Policy, after the Warrant Exercise Closing: (i) the rights of the Warrant Holder Indemnified Parties under this Article 7 shall be the sole and exclusive remedies of the Warrant Holder Indemnified Parties with respect to claims covered by Section 7.1 or otherwise arising under or relating to this Warrant, the Warrant Exercise Closing and the transactions that are contemplated by this Warrant, and neither the Stockholder Representative nor the Company Equityholder Indemnified Parties shall have any Liability to the Warrant Holder Indemnified Parties for any Losses except as expressly provided in Section 7.1, and (ii) the rights of the Company Equityholder Indemnified Parties under this Article 7 shall be the sole and exclusive remedies of the Company Equityholder Indemnified Parties with respect to claims covered by Section 7.2 or otherwise arising under or relating to this Warrant, the Warrant Exercise Closing and the transactions that are contemplated by this Warrant and no Warrant Holder Indemnified Party shall have any Liability to any Company Equityholder Indemnified Party except as expressly provided in Section 7.2. The provisions of this Section 7.8 shall not,

however, prevent or limit a cause of action under Section 9.5. Except as expressly set forth in this Warrant, any information or advice provided by the Warrant Holder to the Company prior to or during the Warrant Period in accordance with Article 7 is made available on an "as is" basis and the Company expressly disclaims any liability with respect to the accuracy, completeness, or compliance with applicable Laws of such information or advice.

ARTICLE 8
TERMINATION

Section 8.1. Termination. This Warrant may be terminated, and the transactions contemplated hereby may be abandoned:

(a) at any time for any reason prior to the Warrant Expiration Date (as it may be extended pursuant to this Warrant), by the Warrant Holder, effective **[****]** after the date written notice of such termination is given by the Warrant Holder to the Company;

(b) at any time after the Warrant Expiration Date (as it may be extended pursuant to this Warrant) but prior to the Warrant Exercise Closing, by the Warrant Holder, by giving written notice to the Company, if a condition set forth in Section 2.4(a)(i) or Section 2.4(a)(ii) of this Warrant would not be satisfied at such time, and, if such breach or inaccuracy is capable of being cured, the Company has failed to cure such breach within **[****]** days after written notice of such breach is given to the Company; provided, that the Warrant Holder is not in breach of this Warrant at the time of such termination;

(c) at any time after the Warrant Expiration Date (as it may be extended pursuant to this Warrant) but prior to the Warrant Exercise Closing, by the Company by giving written notice to the Warrant Holder if the Warrant Holder has materially breached this Warrant after the Warrant Expiration Date and if such breach is capable of being cured, the Warrant Holder has failed to cure such breach within **[****]** days after written notice of such breach is given to the Warrant Holder; provided, that the Company is not in breach of this Warrant at the time of such termination;

(d) by the Company, by giving written notice to the Warrant Holder, if the Warrant Holder has breached its obligation to make a payment referenced in Section 2.1(a) (Grant of Warrant) or Section 2.1(b)(iii) (Development Milestone Payments) when due and failed to cure such breach within **[****]** days after written notice of such breach is given to the Warrant Holder; provided, that the Company is not in breach of this Warrant at the time of such termination;

(e) by either Party, by giving written notice to the other Party, if any Legal Restraint having an effect referred to in Section 2.4(a)(v) is in effect and has become final and nonappealable; provided that the Party seeking to terminate has complied in all material respects with its obligations hereunder to use Commercially Reasonable Efforts to resist the imposition of, and if appropriate, have lifted, such Legal Restraint;

(f) by either Party, by giving written notice to the other Party, if the Warrant Holder has given an Exercise Notice but the Warrant Exercise Closing Date has not occurred on or before that date that is **[****]** days after the Warrant Expiration Date; provided that the Party seeking to terminate has complied in all material respects with its obligations hereunder to use Commercially Reasonable Efforts to effect the Warrant Exercise Closing; and

(g) by the Company pursuant to Section 2.8(a).

Except as expressly set forth above, if the Warrant Holder breaches any provision of this Warrant, the Company will not be entitled to terminate this Warrant on account of such breach, and its sole remedies will be damages or, if appropriate, equitable relief.

Section 8.2. Effect of Termination.

(a) If this Warrant is terminated in accordance with this Article 8, this Warrant shall become void and of no further force or effect, except for the provisions of Section 2.8 (to the extent applicable), Section 2.9 (to the extent applicable), Section 5.12, Section 5.17, Section 5.20, Section 5.22, this Section 8.2 and Article 9; provided that nothing in this Section 8.2 shall be deemed to release any Party from any liability for any breach by such Party of the terms and provisions of this Warrant prior to such termination.

(b) If this Warrant is terminated in accordance with this Article 8, nothing in this Warrant gives either party or its present or future Affiliates any right or license to any Intellectual Property of the other Party, except as otherwise provided in Section 5.22.

ARTICLE 9
MISCELLANEOUS

Section 9.1. Notices. All notices, requests, claims, demands, waivers and other communications under this Warrant shall be in writing and shall be by facsimile, courier services or personal delivery to the following addresses, or to such other addresses as shall be designated from time to time by a Party in accordance with this Section 9.1:

(i) If to the Company, addressed to:

Myonexus Therapeutics, Inc.
8000 Walton Parkway
Suite 255
New Albany, OH 43054
Attention: Chief Executive Officer
Email: **[****]**

with a copy to:

Thompson Hine LLP
312 Walnut Street

14th Floor
Cincinnati, OH 45202-4089
Attention: David J. Willbrand
Facsimile No.: (513) 241-4771
Email: David.Willbrand@ThompsonHine.com

(ii) If to the Stockholder Representative, addressed to the Stockholder Representative at the address indicated in the instrument pursuant to which the Stockholder Representative accepts appointment as such.

(iii) If to the Warrant Holder, addressed to:

Sarepta Therapeutics, Inc.
215 First Street, Suite 415
Cambridge, MA 02142
Attention: General Counsel
Facsimile No.: **[****]**

with a copy to:

Ropes & Gray LLP
Prudential Tower
800 Boylston Street
Boston, MA 02199
Attention: Christopher Comeau, Esq.
Facsimile No.: (617) 235-0566

All notices and communications under this Warrant shall be deemed to have been duly given (x) when delivered by hand, if personally delivered, (y) upon receipt when delivered by a courier (such date of receipt being evidenced by the courier's service records) or (z) when sent, if sent by facsimile, with an acknowledgment of successful receipt being produced by the sending facsimile machine.

Section 9.2. Assignment. Neither this Warrant nor any of the rights, interests or obligations hereunder may be assigned, in whole or in part, by operation of Law or otherwise by any of the Parties without the prior written consent of the other Parties, except that the Warrant Holder may assign, in its sole discretion, any or all of its rights, interests and obligations under this Warrant to a parent entity or Subsidiary of the Warrant Holder; provided, that no such assignment shall release the Warrant Holder from its obligations under this Warrant. Subject to the preceding sentence, this Warrant shall be binding upon, inure to the benefit of and be enforceable by, the parties hereto and their respective successors and assigns.

Section 9.3. Consents and Approvals. For any matter under this Warrant requiring the consent or approval of any Party to be valid and binding on the Parties hereto, such consent or approval must be in writing and executed by the Party providing such consent or approval.

Section 9.4. Enforcement.

(a) Except as otherwise expressly provided in Section 6.2 with respect to the JSC, in Section 2.6(g) with respect to the Contingent Payments and in Section 2.7 with respect to the Closing Balance Sheet and the Closing Date Cash and Liabilities Amount, all disputes, issues, controversies or claims between the Parties arising under or with respect to this Warrant or the Redemption Provisions (each, a "Dispute") shall first be referred to the Executive Officer for resolution. The location, format, frequency, duration and conclusion of the discussions between the Executive Officers shall be left to the discretion of the Executive Officers. The Executive Officers shall negotiate in good faith to resolve a Dispute referred to them within **[****]** Business Days (or such other period as the Executive Officers may approve). The Dispute escalation process described in this paragraphs (a), (b) and (c) of this Section 9.4 is referred to as the "Dispute Escalation Process."

(b) If the Executive Officers do not resolve a Dispute referred to them within **[****]** Business Days (or such other period of time as they may have approved), then either Party may notify the other Party in writing that it desires to elevate such Dispute for resolution by nonbinding mediation. The mediation shall be administered by one or more mediators selected by agreement of the Parties using procedures agreed to by them or, if they fail to agree to the mediator(s) and procedures within **[****]** Business Days after submission of the Dispute for resolution pursuant to this Section 9.4(b), by the American Arbitration Association under its Commercial Mediation Procedures. The Parties shall use good faith efforts to resolve the Dispute through such nonbinding resolution within **[****]** Business Days after submission of the Dispute for resolution pursuant to this Section 9.4(b) (the last day of such **[****]** Business Day period is referred to as the "Conclusion of the Escalation Process").

(c) Notwithstanding anything else in this Warrant or the Redemption Provisions to the contrary, and except as provided below in this Section 9.4(c), the Parties shall participate in the Escalation Process until the Conclusion of the Escalation Process, and shall not terminate negotiations concerning resolution of the matters in Dispute until the earlier of the Conclusion of the Escalation Process or expiration or termination of this Warrant (so long as termination of this Warrant is not the subject of the Dispute). No Party shall commence a lawsuit or seek other remedies with respect to the Dispute (including termination of this Warrant) prior to the Conclusion of the Escalation Process, provided that either party may institute formal legal proceedings at any time: (i) to avoid the expiration of any applicable statute of limitations period, (ii) to preserve a superior position with respect to other creditors, or (iii) to seek an injunction to prevent irreparable harm.

(d) Each Party irrevocably submits to the exclusive jurisdiction of the Chancery Court of the State of Delaware and any state appellate court therefrom within the State of Delaware, or in the event (but only in the event) that such court does not have subject matter jurisdiction over such action or proceeding, in any state or federal court within the State of Delaware, for the purposes of any suit, action or other proceeding arising out of this Warrant or any transaction contemplated hereby. Each Party agrees to commence any such action, suit or proceeding either in the Chancery Court of the State of Delaware and any state appellate court therefrom within the State of Delaware, or in the event (but only in the event) that such court

does not have subject matter jurisdiction over such action or proceeding, in any state or federal court within the State of Delaware. Each Party further agrees that service of any process, summons, notice or document by the U.S. registered mail to such Party's respective address set forth above shall be effective service of process for any action, suit or proceeding in Delaware with respect to any matters to which it has submitted to jurisdiction in this Section 9.4. Each Party irrevocably and unconditionally waives any objection to the laying of venue of any action, suit or proceeding arising out of this Warrant or the transactions contemplated hereby in the Chancery Court of the State of Delaware and any state appellate court therefrom within the State of Delaware, and in the event (but only in the event) that such court does not have subject matter jurisdiction over such action or proceeding, in any state or federal court within the State of Delaware, and hereby and thereby further irrevocably and unconditionally waives and agrees not to plead or claim in any such court that any such action, suit or proceeding brought in any such court has been brought in an inconvenient forum.

(e) EACH PARTY HERETO WAIVES ITS RIGHT TO TRIAL OF ANY ISSUE BY JURY. Each Party hereto (i) certifies that no Representative of any other Party has represented, expressly or otherwise, that such Party would not, in the event of any action, suit or proceeding, seek to enforce the foregoing waiver and (ii) acknowledges that it and the other Parties hereto have been induced to enter into this Warrant, by, among other things, the mutual waiver and certifications in this Section 9.4.

(f) Each Party hereto waives any claim to punitive, exemplary or multiplied damages from the other.

Section 9.5. Specific Enforcement. The Parties agree that irreparable damage would occur and that a Party would not have any adequate remedy at law in the event that any of the provisions of this Warrant were not performed in accordance with their specific terms or were otherwise breached by the other Party. It is accordingly agreed that each Party shall be entitled to an injunction or injunctions to prevent breaches of this Warrant by the other Party and to enforce specifically the terms and provisions of this Warrant in the Chancery Court of the State of Delaware and any state appellate court therefrom within the State of Delaware, or in the event (but only in the event) that such court does not have subject matter jurisdiction over such action or proceeding, in any state or federal court within the State of Delaware, this being in addition to any other remedy to which the Party is entitled at law or in equity and as further set forth in this Article 9.

Section 9.6. Waivers; Amendments.

(a) No failure or delay on the part of any Party in exercising any right, power or remedy hereunder shall operate as a waiver thereof, nor shall any single or partial exercise of any such right, power or remedy preclude any other or further exercise thereof or the exercise of any other right, power or remedy. Except as expressly set forth in Article 7, the remedies provided for herein are cumulative and are not exclusive of any remedies that may be available to any Party at Law, in equity or otherwise.

(b) Except as otherwise specifically set forth in this Warrant, this Warrant may not be amended except by an instrument in writing signed on behalf of each of the Parties and, following the Warrant Exercise Closing Date, the Stockholder Representative.

(c) Except as otherwise specifically set forth in this Warrant, any waiver of any provision of this Warrant shall be effective (i) only if it is made or given in writing and signed by the Warrant Holder and the Company (or, following the Warrant Exercise Closing Date, the Stockholder Representative) or, in the case of a waiver, by the Party granting the waiver and (ii) only in the specific instance and for the specific purpose for which made or given.

Section 9.7. Entire Agreement. This Warrant, the Confidentiality Agreement and the Redemption Provisions contain the entire agreement and understanding between the Parties with respect to the subject matter hereof and thereof and supersede all prior Contracts, both written and oral, relating to such subject matter.

Section 9.8. No Third-Party Beneficiaries. Except as otherwise provided in this Warrant (including in Section 5.21), this Warrant is for the sole benefit of the Parties and their permitted successors and assigns and nothing herein expressed or implied shall give or be construed to give to any Person, other than the Parties and such successors and assigns, any legal or equitable rights hereunder. No covenant or other undertakings in this Warrant shall constitute an amendment to any Plan, program, policy or arrangement, and any covenant or undertaking that suggests that a Plan, program, policy or arrangement will be amended shall be effective only upon the adoption of a written amendment in accordance with the amendment procedures of such Plan, program, policy or arrangement.

Section 9.9. Counterparts. This Warrant may be executed in any number of counterparts and by the Parties in separate counterparts, each of which when so executed shall be deemed to be an original and all of which taken together shall constitute one and the same agreement.

Section 9.10. Governing Law. THIS WARRANT SHALL BE GOVERNED BY, AND CONSTRUED IN ACCORDANCE WITH, THE SUBSTANTIVE LAW OF THE STATE OF DELAWARE, REGARDLESS OF THE LAWS THAT MIGHT OTHERWISE GOVERN UNDER APPLICABLE PRINCIPLES OF CONFLICTS OF LAWS THEREOF.

Section 9.11. Severability. Any term or provision of this Warrant that is invalid or unenforceable in any situation in any jurisdiction shall not affect the validity or enforceability of the remaining terms and provisions hereof or the validity or enforceability of the offending term or provision in any other situation or in any other jurisdiction.

Section 9.12. Stockholder Rights. This Warrant shall not entitle the Warrant Holder, prior to the Warrant Exercise Closing, to any rights as a stockholder of the Company.

[****] = Portions of this exhibit have been omitted pursuant to a confidential treatment request. An unredacted version of this exhibit has been filed separately with the Securities and Exchange Commission.

IN WITNESS WHEREOF, the Company and the Warrant Holder have each caused this Warrant to be duly executed by its authorized signatory as of the day and year first above written.

The Company:
MYONEXUS THERAPEUTICS, INC.
a Delaware corporation

By:	/s/ Michael D. Triplett II
Name:	Michael D. Triplett II
Title:	President & CEO

[Signature Page to Warrant]

The Warrant Holder:
SAREPTA THERAPEUTICS, INC.
a Delaware corporation

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

[Signature Page to Warrant]

EXHIBIT 10.1

SAREPTA THERAPEUTICS, INC.

2018 EQUITY INCENTIVE PLAN

1. Purposes of the Plan. The purposes of the Plan are to:

- attract and retain the best available personnel for positions of substantial responsibility,
- provide additional incentives to Employees, Directors and Consultants, and
- promote the success of the Company's business.

The Plan permits the grant of Incentive Stock Options, Nonstatutory Stock Options, Stock Appreciation Rights, Restricted Stock, Restricted Stock Units, Performance Units, Performance Shares and Performance-Based Cash Awards.

2. Definitions. As used herein, the following definitions will apply:

(a) "Administrator" means the Board or any of its Committees as will be administering the Plan, in accordance with Section 4 of the Plan.

(b) "Affiliate" means any corporation or any other entity (including, but not limited to, partnerships and joint ventures) controlling, controlled by, or under common control with the Company.

(c) "Applicable Laws" means the requirements relating to the administration of equity-based awards under U.S. state corporate laws, U.S. federal and state securities laws, the Code, any stock exchange or quotation system on which the Common Stock is listed or quoted, and the applicable laws of any foreign country or jurisdiction where Awards are, or will be, granted under the Plan.

(d) "Award" means, individually or collectively, a grant under the Plan of Options, Stock Appreciation Rights, Restricted Stock, Restricted Stock Units, Performance Units, Performance Shares or a Performance-Based Cash Award.

(e) "Award Agreement" means the written or electronic agreement setting forth the terms and provisions applicable to each Award granted under the Plan. The Award Agreement is subject to the terms and conditions of the Plan.

(f) "Board" means the Board of Directors of the Company.

(g) "Change in Control" means the occurrence of any of the following events:

(i) Change in Ownership of the Company. A change in the ownership of the Company which occurs on the date that any one person, or more than one person acting as a group ("Person"), acquires ownership of the stock of the Company that, together with the stock held by such Person, constitutes more than fifty percent (50%) of the total voting power of the stock of the Company; provided, however, that for purposes of this subsection (i), the acquisition of additional stock by any one Person who is considered to own more than fifty percent (50%) of the total voting power of the stock of the Company will not be considered a Change in Control; or

(ii) Change in Effective Control of the Company. If the Company has a class of securities registered pursuant to Section 12 of the Exchange Act, a change in the effective control of the Company which occurs on the date that a majority of members of the Board is replaced during any twelve (12) month period by Directors whose appointment or election is not endorsed by a majority of the members of the Board prior to the date of the appointment or election. For purposes of this subsection (ii), if any Person is considered to be in effective control of the Company, the acquisition of additional control of the Company by the same Person will not be considered a Change in Control; or

(iii) Change in Ownership of a Substantial Portion of the Company's Assets. A change in the ownership of a substantial portion of the Company's assets which occurs on the date that any Person acquires (or has acquired during the twelve (12) month period ending on the date of the most recent acquisition by such person or persons) assets from the Company that have a total gross fair market value equal to or more than fifty percent (50%) of the total gross fair market value of all of the assets of the Company immediately prior to such acquisition or acquisitions; provided, however, that for purposes of this subsection (iii), the following will not constitute a change in the ownership of a substantial portion of the Company's assets: (A) a transfer to an entity that is controlled by the Company's stockholders immediately after the transfer; or (B) a transfer of assets by the Company to (1) a stockholder of the Company (immediately before the asset transfer) in exchange for or with respect to the Company's stock, (2) an entity, fifty percent (50%) or more of the total value or voting power of which is owned, directly or indirectly, by the Company, (3) a Person that owns, directly or indirectly, fifty percent (50%) or more of the total value or voting power of all the outstanding stock of the Company, or (4) an entity, at least fifty percent (50%) of the total value or voting power of which is owned, directly or indirectly, by a Person described in this subsection (iii)(B)(3). For purposes of this subsection (iii), gross fair market value means the value of the assets of the Company, or the value of the assets being disposed of, determined without regard to any liabilities associated with such assets.

For purposes of this definition, persons will be considered to be acting as a group if they are owners of a corporation that enters into a merger, consolidation, purchase or acquisition of stock, or similar business transaction with the Company. Further and for the avoidance of doubt, a transaction shall not constitute a Change in Control if: (i) its sole purpose is to change the state of the Company's incorporation, or (ii) its sole purpose is to create a holding company that shall be owned in substantially the same proportions by the persons who held the Company's securities immediately before such transaction.

Notwithstanding the foregoing, for an Award that provides for payment or settlement triggered upon a Change in Control and that constitutes an Award subject to Section 409A of the Code, the foregoing definition shall apply for purposes of vesting of such Award, provided that for purposes of payment or settlement of such Award, such Award shall not be paid or otherwise settled until the earliest of (A) the Participant's "separation from service" within the meaning of Section 409A of the Code, (B) the Participant's death or "disability" within the meaning of Section 409A of the Code or (C) a transaction that qualifies as a change in control event within the meaning of Section 409A of the Code.

(h) "Code" means the Internal Revenue Code of 1986, as amended. Reference to a specific section of the Code or Treasury Regulation thereunder will include such section or regulation, any valid regulation or other official applicable guidance promulgated under such section, and any comparable provision of any future legislation or regulation amending, supplementing, or superseding such section or regulation.

(i) "Committee" means a committee of Directors or of other individuals satisfying Applicable Laws appointed by the Board subject to and in accordance with Section 4 hereof.

(j) "Common Stock" means the common stock of the Company, as adjusted in accordance with Section 15(a) of the Plan.

(k) "Company" means Sarepta Therapeutics, Inc., a Delaware corporation, or any successor thereto.

(l) "Consultant" means any person, including an advisor, engaged by the Company or a Parent or a Subsidiary to render services to such entity other than as an Employee.

(m) "Director" means a member of the Board.

(n) "Disability" means total and permanent disability as defined in Section 22(e)(3) of the Code, provided that in the case of Awards other than Incentive Stock Options, the Administrator in its discretion may determine whether a permanent and total disability exists in accordance with uniform and non-discriminatory standards adopted by the Administrator from time to time. Notwithstanding the foregoing, for an Award that provides for payment or settlement triggered upon a Disability and that constitutes an Award subject to Section 409A of the Code, the foregoing definition shall apply for purposes of vesting of such Award, provided that for

purposes of payment or settlement of such Award, such Award shall not be paid (or otherwise settled) until the earliest of: (A) the Participant's "disability" within the meaning of Section 409A(a)(2)(C)(i) or (ii) of the Code; (B) the Participant's "separation from service" within the meaning of Section 409A of the Code; (C) the date such Award would otherwise be settled pursuant to the terms of the Award agreement; (D) a transaction that qualifies as a change in control event within the meaning of Section 409A of the Code; or (E) death of the Participant.

(o) "Employee" means any person, including Officers and Directors, employed by the Company or any Parent or Subsidiary of the Company. Neither service as a Director nor payment of a director's fee by the Company will be sufficient to constitute "employment" by the Company.

(p) "Exchange Act" means the Securities Exchange Act of 1934, as amended.

(q) "Fair Market Value" means, as of any date, the value of Common Stock as follows:

(i) If the Common Stock is listed on any established stock exchange or a national market system, including without limitation the Nasdaq Global Market, the Nasdaq Global Select Market or the Nasdaq Capital Market, its Fair Market Value shall be the closing sales price for such stock (or, if no closing sales price was reported on that date, as applicable, on the last trading date such closing sales price is reported) as quoted on such exchange or system on the day of determination, as reported in *The Wall Street Journal* or such other source as the Administrator deems reliable;

(ii) If the Common Stock is regularly quoted by a recognized securities dealer but selling prices are not reported, its Fair Market Value shall be the mean of the high bid and low asked prices for the Common Stock on the day of determination (or, if no bids and asks were reported on that date, as applicable, on the last trading date such bids and asks are reported); or

(iii) In the absence of an established market for the Common Stock, the Fair Market Value will be determined in good faith by the Administrator, determined in accordance with Section 409A of the Code.

(r) "Family Member" means any child, stepchild, grandchild, parent, stepparent, grandparent, spouse, former spouse, sibling, niece, nephew, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law, or sister-in-law (including adoptive relationships) of the Employee; any person sharing the Employee's household (other than a tenant or employee); a trust in which these persons (or the Employee) have more than 50% of the beneficial interest; a foundation in which these persons (or the Employee) control the management of assets; and any other entity in which these persons (or the Employee) own more than 50% of the voting interests.

(s) "Fiscal Year" means the fiscal year of the Company.

(t) "Full Value Award" shall mean any Award, other than an Option or a Stock Appreciation Right, that is settled by the issuance of Shares.

(u) "Incentive Stock Option" means an Option that by its terms qualifies and is otherwise intended to qualify as an incentive stock option within the meaning of Section 422 of the Code and the regulations promulgated thereunder.

(v) "Inside Director" means a Director who is an Employee.

(w) "Nonstatutory Stock Option" means an Option that by its terms does not qualify or is not intended to qualify as an Incentive Stock Option.

(x) "Officer" means a person who is an officer of the Company within the meaning of Section 16 of the Exchange Act and the rules and regulations promulgated thereunder.

(y) "Option" means a stock option granted pursuant to the Plan.

(z) "Outside Director" means a Director who is not an Employee.

(aa) "Parent" means a "parent corporation," whether now or hereafter existing, as defined in Section 424(e) of the Code. An entity shall be a Parent of the Company only for such periods as the requisite ownership relationship is maintained, unless otherwise determined by the Administrator.

(bb) "Participant" means the holder of an outstanding Award.

(cc) "Performance-Based Cash Award" means a cash Award pursuant to Section 10 that is payable or otherwise based on the attainment of certain pre-established performance goals during a Performance Period.

(dd) "Performance Goals" will have the meaning set forth in Section 11 of the Plan.

(ee) "Performance Period" means any Fiscal Year of the Company or such other period as determined by the Administrator in its sole discretion.

(ff) "Performance Share" means an Award denominated in Shares which may be earned in whole or in part upon attainment of performance goals or other vesting criteria as the Administrator may determine pursuant to Section 10.

(gg) "Performance Unit" means an Award which may be earned in whole or in part upon attainment of performance goals or other vesting criteria as the Administrator may determine and which may be settled for cash, Shares or other securities, or a combination of the foregoing pursuant to Section 10.

(hh) "Period of Restriction" means the period during which the transfer of Shares of Restricted Stock is subject to restrictions and, therefore, the Shares are subject to a substantial risk of forfeiture. Such restrictions may be based on the passage of time, the achievement of target levels of performance, the occurrence of other events or the combination of any of the foregoing, as determined by the Administrator.

(ii) "Plan" means this 2018 Equity Incentive Plan, as may be amended from time to time.

(jj) "Recoupment Policy" means the Company's Incentive Compensation Recoupment Policy, adopted as of April 27, 2016 and as effective from time to time.

(kk) "Restricted Stock" means Shares issued pursuant to a Restricted Stock award under Section 8 of the Plan, or issued pursuant to the early exercise of an Option.

(ll) "Restricted Stock Unit" means a bookkeeping entry representing an amount equal to the Fair Market Value of one Share, granted pursuant to Section 9. Each Restricted Stock Unit represents an unfunded and unsecured obligation of the Company.

(mm) "Rule 16b-3" means Rule 16b-3 of the Exchange Act or any successor to Rule 16b-3, as in effect when discretion is being exercised with respect to the Plan.

(nn) "Service Provider" means an Employee, Director or Consultant.

(oo) "Share" means a share of the Common Stock, as adjusted in accordance with Section 15(a) of the Plan.

(pp) "Stock Appreciation Right" means an Award, granted alone or in connection with an Option, that pursuant to Section 7 is designated as a Stock Appreciation Right.

(qq) "Stock Ownership Guidelines" means the Company's Stock Ownership Guidelines for Non-Employee Directors and Executive Officers, adopted as of April 27, 2016 and as effective from time to time.

(rr) "Subsidiary" means: (i) a "subsidiary corporation," whether now or hereafter existing, as defined in Section 424(f) of the Code; and (ii) any entity, trade or business (including, without limitation, a partnership or limited liability company) that is directly or indirectly controlled 50% or more (whether by ownership of stock, assets or an equivalent ownership interest or voting interest) by the Company. An entity shall be a Subsidiary of the Company only for such periods as the requisite ownership relationship is maintained, unless otherwise determined by the Administrator.

3. Stock Subject to the Plan.

(a) Stock Subject to the Plan. Subject to adjustment pursuant to Section 15(a) of the Plan, the maximum aggregate number of Shares that may be subject to Awards and sold under the Plan is 2,900,000 Shares, plus the number of Shares subject to outstanding awards under the Amended and Restated 2011 Equity Incentive Plan (the "2011 Plan") that expire or otherwise terminate without having been exercised in full, or are forfeited to or repurchased by us, up to a maximum of 1,487,596 Shares; provided, however, that such aggregate number of Shares available for issuance under the Plan shall be reduced by 1.41 shares for each Share delivered in settlement of any Full Value Award and, provided further, that no more than 2,900,000 Shares may be issued upon the exercise of Incentive Stock Options. The Shares may be authorized, but unissued, or reacquired Common Stock.

(b) Lapsing Awards. If any Award that is not a Full Value Award is forfeited or expires, or such Award is settled for cash (in whole or in part), the Shares subject to such Award shall, to the extent of such forfeiture, expiration or cash settlement, again be available for future grants of Awards under the Plan. To the extent that a Full Value Award is forfeited or expires, or such Full Value Award is settled for cash (in whole or in part), the Shares available under the Plan shall be increased by 1.41 Shares subject to such Full Value Award that is forfeited, expired or settled in cash. Notwithstanding anything to the contrary herein, with respect to Stock Appreciation Rights, all Shares subject to a Stock Appreciation Right will cease to be available under the Plan, other than Shares forfeited due to failure to vest which will become available for future grant or sale under the Plan (unless the Plan has terminated). Shares that have actually been issued under the Plan under any Award will not be returned to the Plan and will not become available for future distribution under the Plan; provided, however, that if Shares issued pursuant to Awards of Restricted Stock, Restricted Stock Units, Performance Shares or Performance Units are repurchased by the Company at the original issuance price or are forfeited to the Company due to failure to vest, such Shares will become available for future grant under the Plan. Shares used to pay the exercise or purchase price of an Award and/or to satisfy the tax withholding obligations related to an Option or Stock Appreciation Right will not become available for future grant or sale under the Plan. Shares used to satisfy the tax withholding obligations related to an Award other than an Option or Stock Appreciation Right will become available for future grant or sale under the Plan. Notwithstanding the foregoing and, subject to adjustment as provided in Section 15(a), the maximum number of Shares that may be issued upon the exercise of Incentive Stock Options will equal the aggregate Share number stated in Section 3(a), plus, to the extent allowable under Section 422 of the Code and the Treasury Regulations promulgated thereunder, any Shares that become available for issuance under the Plan under this Section 3(b).

(c) Vesting Limitations on Awards. Notwithstanding any other provision of the Plan to the contrary, Awards shall become vested over a period of not less than one year following the date the Award is made; provided, however, that, notwithstanding the foregoing, (i) the Administrator may provide that such vesting restrictions may lapse or be waived upon the Participant's Disability, retirement or termination of employment or a Change in Control, (ii) such vesting restrictions shall lapse upon the Participant's death while providing services to the Company, and (iii) Awards that result in the issuance of an aggregate of up to 5% of the shares of Common Stock available pursuant to Section 3(a) may be granted to any one or more Participants without respect to such minimum vesting provisions.

(d) Share Reserve. The Company, during the term of this Plan, will at all times reserve and keep available such number of Shares as will be sufficient to satisfy the requirements of the Plan.

(e) Annual Limit on the Value of Outside Director Awards. Notwithstanding any other provision of the Plan to the contrary, the aggregate value of equity-based Awards granted to an Outside Director in respect of any Fiscal Year plus any cash-based compensation granted to an Outside Director under the Plan or otherwise in respect of any Fiscal Year, in each case, solely with respect to the individual's service as an Outside Director, may not exceed $1,000,000 based on the aggregate Fair Market Value (determined as of the date of grant) of any equity-based Award plus the aggregate value (determined as of the date of grant) of any cash-based compensation, except that with respect to the initial Fiscal Year in which an Outside Director commenced service on the Board, such annual limit shall be $1,500,000.

4. Administration of the Plan.

(a) Procedure.

(i) Multiple Administrative Bodies. Different Committees with respect to different groups of Service Providers may administer the Plan.

(ii) Rule 16b-3; Exchange Listing Rules. With respect to the application of this Plan to Service Providers (other than Directors), the Plan will be administered by a Committee of two or more Outside Directors, each of whom is intended to be (A) to the extent required by Rule 16b-3 promulgated under Section 16(b) of the Exchange Act, a "nonemployee director" as defined in Rule 16b-3;; and (ii) an "independent director" as defined under NASDAQ Listing Rules, the NYSE Listed Company Manual or such other applicable stock exchange rule, as applicable and as amended and/or restated from time to time; and (b) with respect to the application of this Plan to Directors, the Board. If for any reason the appointed Committee does not meet the requirements of Rule 16b-3, such noncompliance shall not affect the validity of Awards, grants, interpretations, or other actions of the Administrator.

(iii) Other Administration. Other than as provided above, the Plan will be administered by (A) the Board or (B) a Committee, which committee will be constituted to satisfy Applicable Laws.

(b) Powers of the Administrator. Subject to the provisions of the Plan, and in the case of a Committee, subject to the specific duties delegated by the Board to such Committee, the Administrator will have the authority, in its discretion:

(i) to determine the Fair Market Value;

(ii) to select the Service Providers to whom Awards may be granted hereunder;

(iii) to determine the number of Shares to be covered by each Award granted hereunder;

(iv) to approve forms of Award Agreements for use under the Plan;

(v) to determine the terms and conditions, not inconsistent with the terms of the Plan, of any Award granted hereunder. Such terms and conditions include, but are not limited to, the exercise price, the time or times when Awards may be exercised (which may be based on performance criteria), any vesting acceleration or waiver of forfeiture restrictions, and any restriction or limitation regarding any Award or the Shares relating thereto, based in each case on such factors as the Administrator will determine;

(vi) to construe and interpret the terms of the Plan and Awards granted pursuant to the Plan;

(vii) to prescribe, amend and rescind rules and regulations relating to the Plan, including rules and regulations relating to sub-plans established for the purpose of satisfying applicable foreign laws or for qualifying for favorable tax treatment under applicable foreign laws;

(viii) to modify or amend each Award (subject to Sections 4(e)(i) and 20(c) of the Plan), including but not limited to the discretionary authority to extend the post-termination exercisability period of Awards, but not beyond the scheduled term of an Option and subject to Section 6(b);

(ix) to allow Participants to satisfy withholding tax obligations in such manner as prescribed in Section 16;

(x) to authorize any person to execute on behalf of the Company any instrument required to effect the grant of an Award previously granted by the Administrator;

(xi) to allow a Participant to defer the receipt of the payment of cash or the delivery of Shares that would otherwise be due to such Participant under an Award pursuant to such procedures as the Administrator may determine, in a manner that is intended to be complaint with, or exempt from, Section 409A of the Code; and

(xii) to make all other determinations deemed necessary or advisable for administering the Plan.

(c) Effect of Administrator's Decision. The Administrator's decisions, determinations and interpretations will be final and binding on all Participants and any other holders of Awards.

(d) No Liability. Under no circumstances shall the Company, its Affiliates, its Subsidiaries, its Parents, the Administrator or the Board incur liability for any indirect, incidental, consequential, or special damages (including lost profits) of any form incurred by any person, whether or not foreseeable and regardless of the form of the act in which such a claim may be brought, with respect to the Plan or the Company's, its Affiliates', its Subsidiaries', its Parents', the Administrator's or the Board's roles in connection with the Plan.

(e) Limitations.

(i) Prohibition Against Repricing. Notwithstanding Section 4(b)(viii), except for adjustments made pursuant to Section 15, the Administrator may not: (i) modify or amend an Option or Stock Appreciation Right to reduce the exercise price of such Option or Stock Appreciation Right after it has been granted; or (ii) cancel any outstanding Option or Stock Appreciation Right in exchange for cash or any other Award with a lower exercise price, in each case, unless such action is approved by stockholders prior to such action being taken. Subject to Section 15, the Administrator shall have the authority, without the approval of the stockholders of the Company, to amend any outstanding Award to increase the price per share or to cancel and replace an Award with the grant of an Award having a price per share that is greater than or equal to the price per share on the date of grant.

(ii) Buyout Provisions. The Administrator may at any time offer to buy out for a payment in cash an Option previously granted based on such terms and conditions as the Administrator will establish and communicate to the Participant at the time that such offer is made. Notwithstanding anything contained in this Section 4(e)(ii) to the contrary, the Administrator shall not be allowed to authorize the buyout of underwater Options or Stock Appreciation Rights without the prior consent of the Company's stockholders.

5. Eligibility. Nonstatutory Stock Options, Restricted Stock, Restricted Stock Units, Stock Appreciation Rights, Performance Units, Performance Shares and Performance-Based Cash Awards may be granted to Service Providers. Incentive Stock Options may be granted only to employees of the Company or any Parent or Subsidiary (as defined in Section (2)(rr)(i) of the Plan).

6. Stock Options.

(a) Limitations.

(i) Each Option will be designated in the Award Agreement as either an Incentive Stock Option or a Nonstatutory Stock Option. However, notwithstanding such designation, to the extent that the aggregate Fair Market Value of the Shares with respect to which Incentive Stock Options are exercisable for the first time by the Participant during any calendar year (under all plans of the Company and any Parent or Subsidiary, as defined in Section (2)(rr)(i) of the Plan) exceeds one hundred thousand U.S. dollars ($100,000), such Options will be treated as Nonstatutory Stock Options. For purposes of this Section 6(a), Incentive Stock Options will be taken into account in the order in which they were granted. The Fair Market Value of the Shares will be determined as of the time the Option with respect to such Shares is granted.

(ii) The Administrator will have complete discretion to determine the number of Shares subject to an Option granted to any Participant, provided that during any Fiscal Year, no Participant will be granted an Option covering more than 500,000 Shares. Notwithstanding the limitation in the previous sentence, with respect to the initial Fiscal Year in which he or she commenced service as an Employee, an Employee may be granted Options covering up to an additional 500,000 Shares. The foregoing share limitations will be adjusted proportionately in connection with any change in the Company's capitalization as described in Section 15(a).

(b) Term of Option. The term of each Option will be stated in the Award Agreement; provided, however, that the term will be no more than ten (10) years from the date of grant thereof. In the case of an Incentive Stock Option granted to a Participant who, at the time the Incentive Stock Option is granted, owns stock representing more than ten percent (10%) of the total combined voting power of all classes of stock of the Company or any Parent or Subsidiary (as defined in Section (2)(rr)(i) of the Plan), the term of the Incentive Stock Option will be five (5) years from the date of grant or such shorter term as may be provided in the Award Agreement.

(c) Option Exercise Price and Consideration.

(i) The per Share exercise price for the Shares to be issued pursuant to the exercise of an Option will be determined by the Administrator, but will be no less than one hundred percent (100%) of the Fair Market Value per Share on the date of grant. In addition, in the case of an Incentive Stock Option granted to an employee of the Company or any Parent or Subsidiary (as defined in Section (2)(rr)(i) of the Plan) who, at the time the Incentive Stock Option is granted, owns stock representing more than ten percent (10%) of the voting power of all classes of stock of the Company or any Parent or Subsidiary (as defined in Section (2)(rr)(i) of the Plan), the per Share exercise price will be no less than one hundred ten percent (110%) of the Fair Market Value per Share on the date of grant. Notwithstanding the foregoing provisions of this Section 6(c)(i), Options may be granted with a per Share exercise price of less than one hundred percent (100%) of the Fair Market Value per Share on the date of grant pursuant to a transaction described in, and in a manner consistent with, Code Section 424(a) or 409A, as applicable and Section 15(a) of the Plan.

(ii) Waiting Period and Exercise Dates. At the time an Option is granted, the Administrator will fix the period within which the Option may be exercised (subject to the right to extend such period under Section 4(b)(viii)) and will determine any conditions that must be satisfied before the Option may be exercised.

(iii) Form of Consideration. The Administrator will determine the acceptable form(s) of consideration for exercising an Option, including the method of payment, to the extent permitted by Applicable Laws. In the case of an Incentive Stock Option, the Administrator will determine the acceptable form of consideration at the time of grant. Such consideration to the extent permitted by Applicable Laws may include, but is not limited to:

(1) cash;

(2) check;

(3) other Shares which have a Fair Market Value on the date of surrender equal to the aggregate exercise price of the Shares as to which said Option will be exercised and provided that accepting such Shares, in the sole discretion of the Administrator, will not result in any adverse accounting consequences to the Company;

(4) by net exercise;

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

(6) a reduction in the amount of any Company liability to the Participant, including any liability attributable to the Participant's participation in any Company-sponsored deferred compensation program or arrangement, in each case, in a manner intended to avoid adverse tax consequences to the Participant under Section 409A of the Code;

(7) such other consideration and method of payment for the issuance of Shares to the extent permitted by Applicable Laws; or

(8) any combination of the foregoing methods of payment.

(d) Exercise of Option.

(i) Procedure for Exercise; Rights as a Stockholder. Any Option granted hereunder will be exercisable according to the terms of the Plan and at such times and under such conditions as determined by the Administrator and set forth in the Award Agreement. An Option may not be exercised for a fraction of a Share.

An Option will be deemed exercised when the Company receives: (i) notice of exercise (in such form as the Administrator specifies from time to time) from the person entitled to exercise the Option, and (ii) full payment for the Shares with respect to which the Option is exercised (together with applicable tax withholdings). Full payment

may consist of any consideration and method of payment authorized by the Administrator and permitted by the Award Agreement and the Plan. Shares issued upon exercise of an Option will be issued in the name of the Participant or, if requested by the Participant, in the name of the Participant and his or her spouse. Until the Shares are issued (as evidenced by the appropriate entry on the books of the Company or of a duly authorized transfer agent of the Company), no right to vote or receive dividends or any other rights as a stockholder will exist with respect to the Shares subject to an Option, notwithstanding the exercise of the Option. The Company will issue (or cause to be issued) such Shares promptly after the Option is exercised. No adjustment will be made for a dividend or other right for which the record date is prior to the date the Shares are issued, except as provided in Section 15(a) of the Plan.

Exercising an Option in any manner will decrease the number of Shares thereafter available, both for purposes of the Plan and for sale under the Option, by the number of Shares as to which the Option is exercised.

(ii) Termination of Relationship as a Service Provider. If a Participant ceases to be a Service Provider, other than upon the Participant's termination as the result of the Participant's death or Disability, the Participant may exercise his or her Option within such period of time as is specified in the Award Agreement to the extent that the Option is vested on the date of termination (but in no event later than the expiration of the term of such Option as set forth in the Award Agreement). In the absence of a specified time in the Award Agreement, the Option will remain exercisable for three (3) months following the Participant's termination. Unless otherwise provided by the Administrator, if on the date of termination the Participant is not vested as to his or her entire Option, the Shares covered by the unvested portion of the Option will revert to the Plan. If after termination the Participant does not exercise his or her Option within the time specified by the Administrator, the Option will terminate, and the Shares covered by such Option will revert to the Plan.

(iii) Disability of Participant. If a Participant ceases to be a Service Provider as a result of the Participant's Disability, the Participant may exercise his or her Option within such period of time as is specified in the Award Agreement to the extent the Option is vested on the date of termination (but in no event later than the expiration of the term of such Option as set forth in the Award Agreement). In the absence of a specified time in the Award Agreement, the Option will remain exercisable for twelve (12) months following the Participant's termination. Unless otherwise provided by the Administrator, if on the date of termination the Participant is not vested as to his or her entire Option, the Shares covered by the unvested portion of the Option will revert to the Plan. If after termination the Participant does not exercise his or her Option within the time specified herein, the Option will terminate, and the Shares covered by such Option will revert to the Plan.

(iv) Death of Participant. If a Participant dies while a Service Provider, then the vesting and exercisability of all shares subject to the Option shall be accelerated as to 100% of the Shares subject to the Option as of such Participant's death. If a Participant dies while a Service Provider, the Option may be exercised following the Participant's death within such period of time as is specified in the Award Agreement to the extent that the Option is vested on the date of death (but in no event may the Option be exercised later than the expiration of the term of such Option as set forth in the Award Agreement), by the Participant's designated beneficiary, provided such beneficiary has been designated prior to Participant's death in a form acceptable to the Administrator. If no such beneficiary has been designated by the Participant, then such Option may be exercised by the personal representative of the Participant's estate or by the person(s) to whom the Option is transferred pursuant to the Participant's will or in accordance with the laws of descent and distribution. In the absence of a specified time in the Award Agreement, the Option will remain exercisable for twelve (12) months following Participant's death. If the Option is not so exercised within the time specified herein, the Option will terminate, and the Shares covered by such Option will revert to the Plan.

(v) Other Termination. A Participant's Award Agreement also may provide that if the exercise of the Option following the termination of Participant's status as a Service Provider (other than upon the Participant's death or Disability) would result in liability under Section 16(b) of the Exchange Act, then the Option will terminate on the earlier of (A) the expiration of the term of the Option set forth in the Award Agreement, or (B) the tenth (10th) day after the last date on which such exercise would result in such liability under Section 16(b) of the Exchange Act. Finally, a Participant's Award Agreement may also provide that if the exercise of the Option following the termination of the Participant's status as a Service Provider (other than upon the Participant's death or Disability) would be prohibited at any time solely because the issuance of Shares would violate the registration requirements under the Securities Act, then the Option will terminate on the earlier of (A) the expiration of the term

of the Option, or (B) the expiration of a period of three (3) months after the termination of the Participant's status as a Service Provider during which the exercise of the Option would not be in violation of such registration requirements.

7. Stock Appreciation Rights.

(a) Grant of Stock Appreciation Rights. Subject to the terms and conditions of the Plan, a Stock Appreciation Right may be granted to Service Providers at any time and from time to time as will be determined by the Administrator, in its sole discretion.

(b) Number of Shares. The Administrator will have complete discretion to determine the number of Stock Appreciation Rights granted to any Participant, provided that during any Fiscal Year, no Participant will be granted Stock Appreciation Rights covering more than 500,000 Shares. Notwithstanding the limitation in the previous sentence, with respect to the initial Fiscal Year in which he or she commenced service as an Employee, an Employee may be granted Stock Appreciation Rights covering up to an additional 500,000 Shares. The foregoing limitations will be adjusted proportionately in connection with any change in the Company's capitalization as described in Section 15(a).

(c) Exercise Price and Other Terms. The Administrator, subject to the provisions of the Plan, will have complete discretion to determine the terms and conditions of Stock Appreciation Rights granted under the Plan, provided, however, that the exercise price will not be less than one hundred percent (100%) of the Fair Market Value of a Share on the date of grant. Notwithstanding the foregoing provisions of this Section 7(c), Stock Appreciation Rights may be granted with a per Share exercise price of less than one hundred percent (100%) of the Fair Market Value per Share on the date of grant pursuant to a transaction described in, and in a manner consistent with, Code Section 424(a) or 409A, as applicable, and Section 15(A) of the Plan. If a Participant dies while a Service Provider, then the vesting and exercisability of all shares subject to the Stock Appreciation Rights shall be accelerated as to 100% of the Shares subject to the Stock Appreciation Rights as of such Participant's death.

(d) Stock Appreciation Right Agreement. Each Stock Appreciation Right grant will be evidenced by an Award Agreement that will specify the exercise price, the term of the Stock Appreciation Right, the acceptable forms of consideration for exercise (which may include any form of consideration permitted by Section 6(c)(iii), the conditions of exercise, and such other terms and conditions as the Administrator, in its sole discretion, will determine.

(e) Expiration of Stock Appreciation Rights. A Stock Appreciation Right granted under the Plan will expire upon the date determined by the Administrator, in its sole discretion, and set forth in the Award Agreement; provided, however, that the term will be no more than ten (10) years from the date of grant thereof. Notwithstanding the foregoing, the rules of Section 6(d) relating to exercise also will apply to Stock Appreciation Rights.

(f) Payment of Stock Appreciation Right Amount. Upon exercise of a Stock Appreciation Right, a Participant will be entitled to receive payment from the Company in an amount determined by multiplying:

(i) The difference between the Fair Market Value of a Share on the date of exercise over the exercise price; by

(ii) The number of Shares with respect to which the Stock Appreciation Right is exercised.

At the discretion of the Administrator, the payment upon Stock Appreciation Right exercise may be in cash, in Shares of equivalent value, or in some combination thereof.

8. Restricted Stock.

(a) Grant of Restricted Stock. Subject to the terms and provisions of the Plan, the Administrator, at any time and from time to time, may grant Shares of Restricted Stock to Service Providers in such amounts as the Administrator, in its sole discretion, will determine.

(b) Restricted Stock Agreement. Each Award of Restricted Stock will be evidenced by an Award Agreement that will specify the Period of Restriction, the number of Shares granted, and such other terms and conditions as the Administrator, in its sole discretion, will determine. Unless the Administrator determines otherwise, the Company as escrow agent will hold Shares of Restricted Stock until the restrictions on such Shares have lapsed. Notwithstanding the foregoing sentence, during any Fiscal Year no Participant will receive more than an aggregate of 100,000 Shares of Restricted Stock. Notwithstanding the foregoing limitation, with respect to the initial Fiscal Year in which he or she commenced service as an Employee, an Employee may be granted an aggregate of up to an additional 100,000 Shares of Restricted Stock. The foregoing limitations will be adjusted proportionately in connection with any change in the Company's capitalization as described in Section 15(a).

(c) Transferability. Except as provided in this Section 8, Shares of Restricted Stock may not be sold, transferred, pledged, assigned, or otherwise alienated or hypothecated until the end of the applicable Period of Restriction.

(d) Other Restrictions. The Administrator, in its sole discretion, may impose such other restrictions on Shares of Restricted Stock as it may deem advisable or appropriate.

(e) Removal of Restrictions. Except as otherwise provided in this Section 8, Shares of Restricted Stock covered by each Restricted Stock grant made under the Plan will be released from escrow as soon as practicable after the last day of the Period of Restriction or at such other time as the Administrator may determine. The Administrator, in its sole discretion, may reduce or waive any restrictions for such Award and may accelerate the time at which any restrictions will lapse or be removed.

(f) Voting Rights. During the Period of Restriction, Service Providers holding Shares of Restricted Stock granted hereunder may exercise full voting rights with respect to those Shares, unless the Administrator determines otherwise.

(g) Dividends and Other Distributions. Except as otherwise provided below, during the Period of Restriction, Service Providers holding Shares of Restricted Stock will be entitled to receive all dividends and other distributions paid with respect to such Shares, unless the Administrator provides otherwise. If any such dividends or distributions are paid in Shares, the Shares will be subject to the same restrictions on transferability and forfeitability as the Shares of Restricted Stock with respect to which they were paid. In addition, regardless of whether they are paid in cash or in Shares, all dividends and other distributions which are paid with respect to a Share of Restricted Stock before it vests shall be subject to the vesting conditions of the corresponding Award of Restricted Stock and will only be paid out to the Participant to the extent that the vesting conditions are subsequently satisfied and the share of Restricted Stock vests.

(h) Return of Restricted Stock to Company. On the date set forth in the Award Agreement, the Restricted Stock for which restrictions have not lapsed will revert to the Company and again will become available for grant under the Plan.

(i) Performance Restrictions. The Administrator, in its sole discretion, may set restrictions based upon the achievement of Performance Goals. The Performance Goals will be set by the Administrator at the time it determines in its sole discretion, provided that the achievement of the Performance Goals is substantially uncertain to be attained (as determined in the sole discretion of the Administrator).

9. Restricted Stock Units.

(a) Grant. Restricted Stock Units may be granted at any time and from time to time as determined by the Administrator. After the Administrator determines that it will grant Restricted Stock Units under the Plan, it will advise the Participant in an Award Agreement of the terms, conditions, and restrictions related to the

grant, including the number of Restricted Stock Units. Notwithstanding anything to the contrary in this subsection (a), for Restricted Stock Units granted subject to restrictions based upon the achievement of Performance Goals, during any Fiscal Year of the Company, no Participant will receive more than an aggregate of 100,000 Restricted Stock Units. Notwithstanding the limitation in the previous sentence, with respect to the initial Fiscal Year in which he or she commenced service as an Employee, an Employee may be granted an aggregate of up to an additional 100,000 Restricted Stock Units. The foregoing limitations will be adjusted proportionately in connection with any change in the Company's capitalization as described in Section 15(a).

(b) Vesting Criteria and Other Terms. The Administrator will set vesting criteria in its discretion, which, depending on the extent to which the criteria are met, will determine the number of Restricted Stock Units that will be paid out to the Participant. The Administrator may set vesting criteria based upon the achievement of Company-wide, business unit, or individual goals (including, but not limited to, continued employment), or any other basis determined by the Administrator in its discretion.

(c) Earning Restricted Stock Units. Upon meeting the applicable vesting criteria, the Participant will be entitled to receive a payout as determined by the Administrator. Notwithstanding the foregoing, at any time after the grant of Restricted Stock Units, the Administrator, in its sole discretion, may reduce or waive any vesting criteria that must be met to receive a payout and may accelerate the time at which any restrictions will lapse or be removed.

(d) Form and Timing of Payment. Payment of earned Restricted Stock Units will be made as soon as practicable after the date(s) set forth in the Award Agreement or as otherwise provided in the applicable Award Agreement or as required by Applicable Laws. The Administrator, in its sole discretion, may pay earned Restricted Stock Units in cash, Shares, or a combination thereof. Shares represented by Restricted Stock Units that are fully paid in cash again will not reduce the number of Shares available for grant under the Plan.

(e) Cancellation. On the date set forth in the Award Agreement, all unearned Restricted Stock Units will be forfeited to the Company.

(f) Performance Restrictions. The Administrator, in its sole discretion, may set restrictions based upon the achievement of Performance Goals. The Performance Goals will be set by the Administrator at the time it determines in its sole discretion, provided that the achievement of the Performance Goals is substantially uncertain to be attained (as determined in the sole discretion of the Administrator).

(g) Dividend Equivalents. In the event that the Administrator decides in its sole discretion to credit dividends (whether in cash or shares) with respect to Restricted Stock Units, all dividend equivalents and other deemed distributions that are credited prior to payment under such Award, will be subject to the same restrictions on transferability and forfeitability as the Restricted Stock Units with respect to which they are credited. Further, the vesting conditions of such amounts will only be paid out to the Participant to the extent that the vesting conditions are subsequently satisfied and the corresponding Restricted Stock Unit vests.

10. Performance Units, Performance Shares and Performance Based Cash Awards.

(a) Grant of Performance Units/Shares. Performance Units and Performance Shares may be granted to Service Providers at any time and from time to time, as will be determined by the Administrator, in its sole discretion. The Administrator will have complete discretion in determining the number of Performance Units and Performance Shares granted to each Participant, provided that during any Fiscal Year, for Performance Units or Performance Shares granted subject to restrictions based upon the achievement of Performance Goals, (i) no Participant will receive Performance Units having an initial value greater than $3,250,000, and (ii) no Participant will receive more than 250,000 Performance Shares. Notwithstanding the foregoing limitation, for Performance Shares granted subject to restrictions based upon the achievement of Performance Goals, with respect to the initial Fiscal Year in which he or she commenced service as a Service Provider, a Service Provider may be granted up to an additional 250,000 Performance Shares and additional Performance Units having an initial value up to $3,250,000. The foregoing limitations will be adjusted proportionately in connection with any change in the Company's capitalization as described in Section 15(a).

(b) Value of Performance Units/Shares. Each Performance Unit will have an initial value that is established by the Administrator on or before the date of grant. Each Performance Share will have an initial value equal to the Fair Market Value of a Share on the date of grant.

(c) Performance Objectives and Other Terms. The Administrator will set performance objectives or other vesting provisions (including, without limitation, continued status as a Service Provider) in its sole discretion which, depending on the extent to which they are met, will determine the number or value of Performance Units/Shares that will be paid out to the Service Providers. The time period during which the performance objectives or other vesting provisions must be met will be called the "Performance Period." Each Award of Performance Units/Shares will be evidenced by an Award Agreement that will specify the Performance Period, and such other terms and conditions as the Administrator, in its sole discretion, will determine. The Administrator may set performance objectives based upon the achievement of Company-wide, divisional, or individual goals, applicable federal or state securities laws, or any other basis determined by the Administrator in its sole discretion.

(d) Earning of Performance Units/Shares. The holder of Performance Units/Shares will be entitled to receive a payout of the number of Performance Units/Shares earned by the Participant during the Performance Period, to be determined as a function of the extent to which the corresponding performance objectives or other vesting provisions have been achieved. After the grant of a Performance Unit/Share, the Administrator, in its sole discretion, may reduce or waive any performance objectives or other vesting provisions for such Performance Unit/Share and may accelerate the time at which any restrictions will lapse or be removed.

(e) Form and Timing of Payment of Performance Units/Shares. Payment of earned Performance Units/Shares will be made (i) upon achievement of strategic and/or operational goals prior to the expiration of the applicable Performance Period, at the discretion of the Administrator or (ii) as soon as practicable after the expiration of the applicable Performance Period, or as otherwise provided in the applicable Award Agreement or as required by Applicable Laws. The Administrator, in its sole discretion, may pay earned Performance Units/Shares in the form of cash, in Shares (which have an aggregate Fair Market Value equal to the value of the earned Performance Units/Shares at the close of the applicable Performance Period (or on the payment date pursuant to Section 10(e)(i) above)) or in a combination thereof.

(f) Cancellation of Performance Units/Shares. On the date set forth in the Award Agreement, all unearned or unvested Performance Units/Shares will be forfeited to the Company, and again will be available for grant under the Plan.

(g) Performance-Based Cash Awards. Performance-Based Cash Awards may be granted either alone or in addition to or in tandem with other Awards granted under this Plan. Subject to the provisions of this Plan, the Administrator shall have authority to determine, in its sole discretion, the Service Providers to whom, and the time or times at which, Performance-Based Cash Awards shall be made, the dollar amount to be awarded pursuant to such Performance-Based Cash Award, and all other conditions for the payment of the Performance-Based Cash Award. The Administrator may also provide for the payment of a dollar amount under a Performance-Based Cash Award upon the completion of a specified Performance Period.

Except as otherwise provided herein or as otherwise determined by the Administrator, the Administrator shall condition the right to payment of any Performance-Based Cash Award upon the attainment of specified performance criteria (including, the Performance Goals specified in Section 11(b) below) established pursuant to Section 11(c) below and such other factors as the Administrator may determine in its sole discretion

Subject to Section 11(c), for any Participant, the Administrator may specify a targeted Performance-Based Cash Award for a Performance Period (each an "Individual Target Award"). An Individual Target Award may be expressed as a fixed dollar amount, a percentage of the Participant's base pay, as a percentage of a bonus pool funded by a formula as determined by the Administrator in its sole discretion based on achievement of Performance Goals, or an amount determined pursuant to an objective formula or standard. The Administrator's establishment of an Individual Target Award for a Participant for a Performance Period shall not imply or require that the same level or any Individual Target Award be established for the Participant for any subsequent Performance Period or for or any other Participant for that Performance Period or any subsequent Performance Period. At the time the Performance Goals are established (as provided in Section 11(c)), the Administrator shall prescribe a formula to be

used to determine the maximum and/or threshold percentages (which may be greater or less than one-hundred percent (100%), as applicable) of an Individual Target Award that may be earned or payable based upon the degree of attainment of the Performance Goals during the Performance Period. Notwithstanding anything else herein, unless otherwise specified by the Administrator with respect to an Individual Target Award, the Administrator may elect to pay a Participant an amount that is less than the Participant's Individual Target Award (or attained percentages thereof) regardless of the degree of attainment of the Performance Goals; provided that, except as otherwise specified by the Administrator with respect to an Individual Target Award, no discretion to reduce a Performance-Based Cash Award earned based on achievement of the applicable Performance Goals shall be permitted for any Performance Period in which a Change in Control occurs, or during such Performance Period with regard to the prior Performance Periods if the Performance-Based Cash Awards for the prior Performance Periods have not been paid by the time of the Change in Control, with regard to individuals who were Participants at the time of the Change in Control.

(h) Terms and Conditions of Performance-Based Cash Awards.

(i) Certification. At the expiration of the applicable Performance Period, the Administrator shall determine, in its sole discretion, and certify in writing the extent to which the Performance Goals established pursuant to Section 11(c) are achieved and, if applicable, the percentage of the Participant's Individual Target Award that has been vested and earned.

(ii) Waiver of Limitation. In the event of the Participant's death or Disability, or in cases of special circumstances, the Administrator, in its sole discretion, may waive in whole or in part any or all of the limitations imposed hereunder (if any) with respect to any or all of a Performance-Based Cash Award.

(iii) Termination. Unless otherwise determined by the Committee in its sole discretion, no Performance-Based Cash Award or pro rata portion thereof shall be payable to any Participant who incurs a termination prior to the date such Performance-Based Cash Award is paid.

(iv) Maximum Payments. In granting Performance-Based Cash Awards, the aggregate amount of compensation to be paid to any one Participant in respect of all Performance-Based Cash Awards granted to such Participant in respect of any one calendar year shall not exceed $10,000,000 per year; provided, however, that with respect to any Performance-Based Cash Awards that are subject to a Performance Period longer or shorter than one year, the foregoing Performance-Based Cash Awards limit shall be proportionately adjusted upward or downward; and provided, further, that any Performance-Based Cash Awards that are cancelled in respect of any period shall be counted against this limit for such period.

(i) Performance Restrictions. The Administrator, in its sole discretion, may set restrictions based upon the achievement of Performance Goals. The Performance Goals will be set by the Administrator at the time it determines in its sole discretion, provided that the achievement of the Performance Goals is substantially uncertain to be attained (as determined in the sole discretion of the Administrator).

11. Performance-Based Compensation.

(a) General. If the Administrator, in its discretion, may grant Awards that are based on Performance Goals or other specific criteria or goals.

(b) Performance Goals. The granting and/or vesting of Awards of Restricted Stock, Restricted Stock Units, Performance Shares, Performance Units and Performance-Based Cash Awards and other incentives under the Plan may be made subject to the attainment of performance goals relating to one or more business criteria and may provide for a targeted level or levels of achievement ("Performance Goals") including: (i) attainment of research and development milestones; (ii) bookings; (iii) business divestitures and acquisitions; (iv) cash flow; (v) cash position; (vi) contract awards or backlog; (vii) customer renewals; (viii) customer retention rates from an acquired company, business unit or division; (ix) earnings (which may include earnings before interest and taxes, earnings before taxes and net earnings); (x) earnings per Share; (xi) expenses; (xii) gross margin; (xiii) growth in shareholder value relative to the moving average of the S&P 500 Index or another index; (xiv) internal rate of return; (xv) market share; (xvi) net income; (xvii) net profit; (xviii) net sales; (xix) new product development; (xx) new product invention or innovation; (xxi) number of customers; (xxii) operating cash flow; (xxiii) operating

expenses; (xxiv) operating income; (xxv) operating margin; (xxvi) overhead or other expense reduction; (xxvii) product defect measures; (xxviii) product release timelines; (xxix) productivity; (xxx) profit; (xxxi) return on assets; (xxxii) return on capital; (xxxiii) return on equity; (xxxiv) return on investment; (xxxv) return on sales; (xxxvi) revenue; (xxxvii) revenue growth; (xxxviii) sales results; (xxxix) sales growth; (xl) stock price; (xli) time to market; (xlii) total shareholder return; and (xliii) working capital. Any criteria used may be (A) measured in absolute terms, (B) measured in terms of growth, (C) compared to another company or companies, (D) measured against the market as a whole and/or according to applicable market indices, (E) measured against the performance of the Company as a whole or a segment of the Company and/or (F) measured on a pre-tax or post-tax basis (if applicable). Further, any Performance Goals may be used to measure the performance of the Company as a whole or a business unit or other segment of the Company, or one or more product lines or specific markets and may be measured relative to a peer group or index. The Performance Goals may differ from Participant to Participant and from Award to Award. The Administrator may determine whether any significant element(s) will be included in or excluded from the calculation of any Performance Goal with respect to any Participant. In all other respects, Performance Goals will be calculated in accordance with the Company's financial statements, generally accepted accounting principles, or under a methodology established by the Administrator prior to the issuance of an Award and which is consistently applied with respect to a Performance Goal in the relevant Performance Period. The Administrator will appropriately adjust any evaluation of performance under a Performance Goal to exclude (1) any items that are unusual in nature or infrequently occurring, or both, within the meaning of FASB Accounting Standards Codification and/or in management's discussion and analysis of financial conditions and results of operations appearing in the Company's annual report to stockholders for the applicable year, or (2) the effect of any changes in accounting principles affecting the Company's or a business unit's reported results, unless the Adminnistrator decides otherwise in its sole discretion. In addition, the Administrator will adjust any performance criteria, Performance Goal, or other feature of an Award that relates to or is wholly or partially based on the number of, or the value of, any stock of the Company, to reflect any stock dividend or split, repurchase, recapitalization, combination, or exchange of shares or other similar changes in such stock.

(c) Procedures. With respect to any Award granted subject to Performance Goals, at the time determined by the Administrator in its sole discretion provided that achievement of the Performance Goals is substantially uncertain to be attained (as determined in the sole discretion of the Administrator), the Administrator will, in writing, (i) designate one or more Participants to whom an Award will be made, (ii) select the Performance Goals applicable to the Performance Period, (iii) establish the Performance Goals, and amounts of such Awards, as applicable, which may be earned for such Performance Period, and (iv) specify the relationship between Performance Goals and the amounts of such Awards, as applicable, to be earned by each Participant for such Performance Period. Following the completion of each Performance Period, the Administrator will certify in writing whether the applicable Performance Goals have been achieved for such Performance Period. In determining the amounts earned by a Participant, the Administrator will have the right to reduce or eliminate (but not to increase) the amount payable at a given level of performance to take into account additional factors that the Administrator may deem relevant to the assessment of individual or corporate performance for the Performance Period. A Participant will be eligible to receive payment pursuant to an Award subject to Performance Goals for a Performance Period only to the extent the Performance Goals for such period are achieved, unless otherwise determined by the Administrator in its sole discretion.

(d) Determination of Amounts Earned. In determining the amounts earned by a Participant pursuant to an Award granted subject to Performance Goals, the Committee will have the right to (i) reduce or eliminate (but not to increase) the amount payable at a given level of performance to take into account additional factors that the Committee may deem relevant to the assessment of individual or corporate performance for the Performance Period, (ii) determine what actual Award, if any, will be paid in the event of a termination of employment as the result of a Participant's death or Disability or upon a Change in Control or in the event of a termination of employment following a Change in Control prior to the end of the Performance Period, and (iii) determine what actual Award, if any, will be paid in the event of a termination of employment other than as the result of a Participant's death or Disability prior to a Change of Control and prior to the end of the Performance Period to the extent an actual Award would have otherwise been achieved had the Participant remained employed through the end of the Performance Period.

12. Compliance With Code Section 409A. Although the Company does not guarantee to a Participant the particular tax treatment of any Award, the Plan and all Awards are intended to be exempt from the application of, or comply with, the requirements of Code Section 409A such that the grant, payment, settlement or deferral will not be subject to the additional tax or interest applicable under Code Section 409A, except as otherwise determined in the sole discretion of the Administrator. To the extent that an Award or payment, or the settlement or deferral thereof, is subject to Code Section 409A the Award will be granted, paid, settled or deferred in a manner intended to meet the requirements of Code Section 409A.

A Participant's termination shall not be deemed to have occurred for purposes of any provision of an Award governed by Code Section 409A providing for payment upon or following a termination of the Participant's employment unless such termination is also a "separation from service" within the meaning of Code Section 409A and, for purposes of any such provision of an Award governed by Code Section 409A, references to a "termination," "termination of employment" or like terms shall mean separation from service. Notwithstanding any provision to the contrary in the Plan or the Award agreement, if the Participant is deemed on the date of the Participant's termination to be a "specified employee" within the meaning of that term under Code Section 409A(a)(2)(B) and using the identification methodology selected by the Company from time to time, or if none, the default methodology set forth in Code Section 409A, then with regard to any such payment, to the extent required to be delayed in compliance with Code Section 409A(a)(2)(B), such payment shall not be made prior to the earlier of (i) the expiration of the six-month period measured from the date of the Participant's separation from service, and (ii) the date of the Participant's death. All payments delayed pursuant to this Section 12 shall be paid to the Participant on the first day of the seventh month following the date of the Participant's separation from service or, if earlier, on the date of the Participant's death.

13. Leaves of Absence/Transfer Between Locations. Unless the Administrator provides otherwise and except as required by Applicable Laws, vesting of Awards granted hereunder will be suspended during any unpaid leave of absence. A Participant will not cease to be an Employee in the case of (i) any leave of absence approved by the Company or (ii) transfers between locations of the Company or between the Company, its Parent, or any Subsidiary. For purposes of Incentive Stock Options, no such leave may exceed three (3) months, unless reemployment upon expiration of such leave is guaranteed by statute or contract. If reemployment upon expiration of a leave of absence approved by the Company is not so guaranteed, then six (6) months following the first (1st) day of such leave, any Incentive Stock Option held by the Participant will cease to be treated as an Incentive Stock Option and will be treated for tax purposes as a Nonstatutory Stock Option.

14. Non-Transferability of Awards. Unless determined otherwise by the Administrator, an Award may not be sold, pledged, assigned, hypothecated, transferred, or disposed of in any manner other than by will or by the laws of descent or distribution and may be exercised, during the lifetime of the Participant, only by the Participant. If the Administrator makes an Award transferable, including, without limitation, by instrument to an inter vivos or testamentary trust in which the Awards are to be passed to beneficiaries upon the death of the trustor (settlor) or by gift to Family Members, such Award will contain such additional terms and conditions as the Administrator deems appropriate.

15. Adjustments; Dissolution or Liquidation; Merger or Change in Control.

(a) Adjustments. In the event that any dividend or other distribution (whether in the form of cash, Shares, other securities, or other property), recapitalization, stock split, reverse stock split, reorganization, merger, consolidation, split-up, spin-off, combination, repurchase, or exchange of Shares or other securities of the Company, or other change in the corporate structure of the Company affecting the Shares occurs, the Administrator, in order to prevent diminution or enlargement of the benefits or potential benefits intended to be made available under the Plan, will adjust the number and class of Shares that may be delivered under the Plan and/or the number, class, and price of Shares covered by each outstanding Award, and the numerical Share limits set forth in Sections 3, 6, 7, 8, 9 and 10 of the Plan.

(b) Dissolution or Liquidation. In the event of the proposed dissolution or liquidation of the Company, the Administrator will notify each Participant as soon as practicable prior to the effective date of such proposed transaction. To the extent it has not been previously exercised, an Award will terminate immediately prior to the consummation of such proposed action.

(c) Change in Control.

(i) In the event of a Change in Control, each outstanding Award will be treated as the Administrator determines without a Participant's consent, including, without limitation, that

(1) Awards will be assumed, or substantially equivalent Awards will be substituted, by the acquiring or succeeding entity (or an affiliate thereof) (each a "successor") with appropriate adjustments as to the number and kind of shares and prices;

(2) upon written notice to a Participant, that the Participant's Awards will terminate upon or immediately prior to the consummation of such Change in Control;

(3) outstanding awards will vest and become exercisable, realizable, or payable, or restrictions applicable to an Award will lapse, in whole or in part prior to or upon consummation of such Change in Control, and, to the extent the Administrator determines, terminate upon or immediately upon effectiveness of such Change in Control;

(4) (A) the termination of an Award in exchange for an amount of cash and/or property, if any, equal to the amount that would have been attained upon the exercise of such Award or realization of the Participant's rights as of the date of the occurrence of the transaction, (and, for the avoidance of doubt, if as of the date of the occurrence of the transaction the Administrator determines in good faith that no amount would have been attained upon the exercise of such Award or realization of the Participant's rights, then such Award may be terminated by the Company without payment), or (B) the replacement of such Award on a substantially equivalent basis with other rights or property selected by the Administrator in its sole discretion; or

(5) any combination of the foregoing. In taking any of the actions permitted under this subsection (c), the Administrator will not be obligated to treat all Awards, all Awards held by a Participant, or all Awards of the same type, similarly.

(ii) In the event that the successor does not assume or substitute for the Award (or portion thereof) on a substantially equivalent basis, the Participant will fully vest in and have the right to exercise all of his or her outstanding Options and Stock Appreciation Rights that are not assumed or substituted for, including Shares as to which such Awards would not otherwise be vested or exercisable, all restrictions on Restricted Stock, Restricted Stock Units, Performance Shares/Units and Performance-Based Cash Awards not assumed or substituted for will lapse, and, with respect to Awards with performance-based vesting not assumed or substituted for, all performance goals or other vesting criteria will be deemed achieved at one hundred percent (100%) of target levels and all other terms and conditions met. In addition, if an Option or Stock Appreciation Right is not assumed or substituted for in the event of a Change in Control, the Administrator will notify the Participant in writing or electronically that the Option or Stock Appreciation Right will be fully vested and exercisable for a period of time determined by the Administrator in its sole discretion, and the Option or Stock Appreciation Right will terminate upon the expiration of such period.

(iii) For the purposes of this subsection (c), an Award will be considered assumed if, following the Change in Control, the Award confers the right to purchase or receive, for each Share subject to the Award immediately prior to the Change in Control, the consideration (whether stock, cash, or other securities or property) or, in the case of a Stock Appreciation Right upon the exercise of which the Administrator determines to pay cash or a Restricted Stock Unit, Performance Share or Performance Unit which the Administrator can determine to pay in cash, the fair market value of the consideration received in the Change in Control by holders of Common Stock for each Share held on the effective date of the transaction (and if holders were offered a choice of consideration, the type of consideration chosen by the holders of a majority of the outstanding Shares); provided, however, that if such consideration received in the Change in Control is not solely common stock of the successor or its Parent, the Administrator may, with the consent of the successor, provide for the consideration to be received upon the exercise of an Option or Stock Appreciation Right or upon the payout of a Restricted Stock Unit, Performance Unit or Performance Share, for each Share subject to such Award (or in the case of an Award settled in cash, the number of implied shares determined by dividing the value of the Award by the per share consideration received by holders of Common Stock in the Change in Control), to be solely common stock of the successor or its Parent equal in fair market value to the per share consideration received by holders of Common Stock in the Change in Control.

(iv) Notwithstanding anything in this subsection (c) to the contrary, an Award that vests, is earned or paid-out upon the satisfaction of one or more performance goals will not be considered assumed if the Company or its successor modifies any of such performance goals without the Participant's consent; provided, however, a modification to such performance goals only to reflect the successor's post-Change in Control corporate structure will not be deemed to invalidate an otherwise valid Award assumption

16. Tax Withholding.

(a) Withholding Requirements. Prior to the delivery of any Shares or cash pursuant to an Award (or exercise thereof), the Company will have the power and the right to deduct or withhold, or require a Participant to remit to the Company, an amount sufficient to satisfy federal, state, local, foreign, or other taxes (including the Participant's FICA obligation) required to be withheld with respect to such Award (or exercise thereof).

(b) Withholding Arrangements. The Administrator, in its sole discretion and pursuant to such procedures as it may specify from time to time, may permit a Participant to satisfy such tax withholding obligation, in whole or in part by (without limitation) (i) paying cash, (ii) electing to have the Company withhold otherwise deliverable cash or Shares having a Fair Market Value equal to the statutory amount required to be withheld, (iii) delivering to the Company already-owned Shares having a Fair Market Value equal to the statutory amount required to be withheld, provided the delivery of such Shares will not result in any adverse accounting consequences as the Administrator determines in its sole discretion, (iv) selling a sufficient number of Shares otherwise deliverable to the Participant through such means as the Administrator may determine in its sole discretion (whether through a broker or otherwise) equal to the amount required to be withheld, or (v) retaining from salary or other amounts payable to the Participant cash having a sufficient value to satisfy the amount required to be withheld. The Fair Market Value of the Shares to be withheld or delivered will be determined as of the date that the taxes are required to be withheld.

17. No Effect on Employment or Service. Neither the Plan nor any Award will confer upon a Participant any right with respect to continuing the Participant's relationship as a Service Provider with the Company, nor will they interfere in any way with the Participant's right or the Company's right to terminate such relationship at any time, with or without cause, to the extent permitted by Applicable Laws.

18. Date of Grant. The date of grant of an Award will be, for all purposes, the date on which the Administrator makes the determination granting such Award, or such other later date as is determined by the Administrator. Notice of the determination will be provided to each Participant within a reasonable time after the date of such grant.

19. Effective Date and Term of Plan. The Plan was adopted by the Board on April 20, 2018, subject to and effective upon approval by stockholders of the Company at the Company's 2018 annual meeting of stockholders as provided in Section 23. The Plan will continue in effect for a term of ten (10) years from the date the Board adopts the Plan, unless terminated earlier under Section 20 of the Plan.

20. Amendment and Termination of the Plan.

(a) Amendment and Termination. The Administrator may at any time amend, alter, suspend or terminate the Plan.

(b) Stockholder Approval. The Company will obtain stockholder approval of any Plan amendment to the extent necessary and desirable to comply with Applicable Laws.

(c) Effect of Amendment or Termination. No amendment, alteration, suspension or termination of the Plan will impair the rights of any Participant, unless mutually agreed otherwise between the Participant and the Administrator, which agreement must be in writing and signed by the Participant and the Company. Termination of the Plan will not affect the Administrator's ability to exercise the powers granted to it hereunder with respect to Awards granted under the Plan prior to the date of such termination.

21. Conditions Upon Issuance of Shares.

(a) Legal Compliance. Shares will not be issued pursuant to the exercise of an Award unless the exercise of such Award and the issuance and delivery of such Shares will comply with Applicable Laws and will be further subject to the approval of counsel for the Company with respect to such compliance.

(b) Investment Representations. As a condition to the exercise of an Award, the Company may require the person exercising such Award to represent and warrant at the time of any such exercise that the Shares are being purchased only for investment and without any present intention to sell or distribute such Shares if, in the opinion of counsel for the Company, such a representation is required.

(c) Recoupment Policy; Stock Ownership Guidelines. All Awards made under the Plan are subject to the Recoupment Policy and the Stock Ownership Guidelines, where applicable.

22. Inability to Obtain Authority. The inability of the Company to obtain authority from any regulatory body having jurisdiction, which authority is deemed by the Company's counsel to be necessary to the lawful issuance and sale of any Shares hereunder, will relieve the Company of any liability in respect of the failure to issue or sell such Shares as to which such requisite authority will not have been obtained.

23. Stockholder Approval. The Plan is subject to approval by the stockholders of the Company at the Company's 2018 annual meeting of stockholders. Such stockholder approval will be obtained in the manner and to the degree required under Applicable Laws.

EXHIBIT 10.2

Sarepta Therapeutics, Inc.

EMPLOYMENT AGREEMENT

EMPLOYMENT AGREEMENT (this "Agreement") between **Sarepta Therapeutics, Inc.**, a Delaware corporation (the "Company"), and **Dr. Gilmore N. O'Neill** (the "Executive"), effective as of June 7, 2018 (the "Effective Date"), subject to and conditioned upon the Executive's commencement of employment with the Company on such date.

W I T N E S S E T H

WHEREAS, the Company desires to employ Executive as a Senior Vice President and the Chief Medical Officer of the Company; and

WHEREAS, the Company and Executive desire to enter into this Agreement and the Change in Control Severance Agreement dated as of May 23, 2018 ("CIC Severance Agreement") to memorialize the terms and conditions of Executive's employment with the Company.

NOW, THEREFORE, in consideration of the foregoing, of the mutual promises contained herein and of other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto hereby agree as follows:

1. POSITION AND DUTIES.

(a) During the Employment Term (as defined in Section 2 hereof), Executive shall serve as Senior Vice President and Chief Medical Officer of the Company. In this capacity, Executive shall have the duties, authorities and responsibilities commensurate with the duties, authorities and responsibilities of persons in similar capacities in similarly-sized companies, and such other duties, authorities and responsibilities as the Chief Executive Officer shall designate from time to time that are not inconsistent with Executive's position as the Chief Medical Officer of the Company. Executive shall report to the Chief Executive Officer of the Company.

(b) During the Employment Term, Executive shall devote all of Executive's business time, energy and skill and the Executive's efforts to the performance of Executive's duties with the Company hereunder, provided that the foregoing shall not prevent Executive from (i) serving on one or more non-profit organization boards of directors, subject to informing and consulting with the Chief Executive Officer; (ii) one or more for-profit company boards of directors, subject to the prior written approval of the Board; (iii) participating in charitable, civic, educational, professional, community or industry affairs; and (iv) managing Executive's passive personal investments so long as such activities, individually or in the aggregate, do not materially interfere or conflict with Executive's duties hereunder or create a potential business or fiduciary conflict of interest.

2. EMPLOYMENT TERM. The Company agrees to employ Executive under and pursuant to the terms of this Agreement, and Executive agrees to be so employed as Senior Vice President and Chief Medical Officer of the Company commencing as of the Effective Date and continuing until Executive's employment is terminated in accordance with Section 8. Executive's employment hereunder may be terminated at any time, subject to the terms in Section 9 hereof. The period of time between the Effective Date and the termination of Executive's employment pursuant to Section 8 or 9 shall be referred to herein as the "Employment Term." Concurrently with the execution of this Agreement, the Company and the Executive shall enter into that certain CIC Severance Agreement. For the avoidance of doubt, the termination of this Agreement shall not operate to terminate the CIC Severance Agreement.

3. BASE SALARY. The Company agrees to pay Executive a base salary at an annual rate of $550,000, payable in accordance with the regular payroll and withholding practices of the Company, but not less frequently than monthly. Executive's Base Salary shall be subject to annual review as part of the Annual Compensation

Review process which typically takes place in the first quarter of the calendar year. Executive's base salary may be increased, but not decreased below its then current level unless the salaries of other senior level executives are reduced in which event the Executive's salary may be decreased in the same percentage as other senior level executives except that the amount of any such reduction shall not exceed more than ten percent (10%) of the Base Salary in effect immediately prior to any such reduction. The base salary as determined in accordance with this Section 3 from time to time shall constitute "Base Salary" for purposes of this Agreement.

4. SIGN-ON BONUS. Subject to Executive's execution of this Agreement, Executive will be entitled to receive a cash sign-on bonus of $379,000, less any required withholdings (the "Sign-On Bonus"). The Company will pay the Sign-On Bonus to Executive on the first payroll period following the earliest of (i) May 23, 2019; (ii) the date that Executive's employment is terminated by the Company without Cause or by Executive for Good Reason, each as defined herein below, or (iii) the date on which a Change of Control occurs (as defined in the CIC Severance Agreement.

5. ANNUAL BONUS. During the Employment Term, Executive shall be eligible to receive an annual discretionary incentive payment under the Company's annual bonus plan as in effect from time to time (the "Annual Bonus") based on a target bonus opportunity of 50% of Executive's Base Salary (the "Target Bonus"), upon the attainment of one or more pre-established performance goals . The pre-established performance goals shall be set by the Company following a good faith consultation with Executive. For calendar year 2018, Executive's Target Bonus will be not less than 50% of Executive's salary paid in calendar year 2018. To the extent determined by the Compensation Committee, all or any portion of Executive's Annual Bonus may be paid in cash or in the form of equity compensation awards under the Company's Amended and Restated 2011 Equity Incentive Plan, as amended and/or restated from time to time, or any successor shareholder-approved Company equity compensation plan. Except as provided in Section 9 below, any portion of the Annual Bonus payable in cash shall be deemed "earned" if the Executive is employed on the last day of the applicable year, and the Annual Bonus, whether paid in cash or equity, shall be paid or delivered no later than March 15th of the calendar year immediately following the applicable year to which the Annual Bonus relates.

6. EQUITY AWARDS.

(a) **RESTRICTED STOCK UNITS.** On the Effective Date, as an inducement material to the Executive entering into this Agreement and thereby becoming an employee of the Company, Executive shall receive an inducement award in the form of 12,000 restricted stock units (the "Restricted Stock Units"). Each Restricted Stock Unit shall represent a contingent right to receive one share of the Company's common stock. The Restricted Stock Units shall be granted pursuant to the 2014 Employment Commencement Incentive Plan, as amended (the "2014 Incentive Plan"). One hundred percent of the Restricted Stock Units shall vest on May 23, 2019. The Restricted Stock Units shall be subject to the terms and conditions contained in the 2014 Incentive Plan and the Company's form of restricted stock agreement under the 2014 Incentive Plan.

(b) **STOCK OPTION**. On the Effective Date, as an inducement material to Executive entering into this Agreement and thereby becoming an employee of the Company, Executive shall be awarded an option to purchase 100,000 incentive shares of the Company's Common Stock (the "Option"). The exercise price of the Option will equal the closing sales price of the Company's Common Stock as reported by The NASDAQ Global Market on the grant date. Twenty-five percent of the shares underlying the Option will vest and become exercisable on the first anniversary of the Effective Date, and thereafter 1/36th of the remaining 75,000 shares underlying the Option will vest and become exercisable on each monthly anniversary of the Effective Date, such that the shares underlying the Option will be fully vested and exercisable on the four-year anniversary of the Effective Date.

(c) **ANNUAL EQUITY AWARDS.** Executive shall be eligible to participate in the Company's annual equity grant program based on Executive's performance, in such amount and form, and subject to such terms and conditions, as established by the Board or the Committee for any such program.

(d) **ACCELERATION.** Notwithstanding the foregoing, any remaining unvested equity awards as of the termination date shall be immediately forfeited and of no further force or effect, except as provided in Section 9(a) or (c)(iv) hereof or in the CIC Severance Agreement.

7. EMPLOYEE BENEFITS.

(a) **BENEFIT PLANS.** Executive shall be entitled to participate in any employee benefit plan that the Company has adopted or may adopt, maintain or contribute to or for the benefit of its executive employees, on the same basis as those benefits are generally made available to other executives of the Company, subject to satisfying the terms and conditions of such plans, including the applicable eligibility requirements.

(b) **VACATIONS.** Executive shall be entitled to paid vacation in accordance with the Company's policy on accrual and use applicable to executive employees as in effect from time to time.

(c) **BUSINESS AND TRAVEL EXPENSES.** Upon presentation of appropriate documentation, Executive shall be reimbursed in accordance with the Company's expense reimbursement policy, for all reasonable business expenses incurred in connection with the performance of the Executive's duties hereunder and the Company's policies with regard thereto.

8. TERMINATION. The Executive's employment and the Employment Term hereunder shall terminate on the first of the following to occur:

(a) **DISABILITY.** Upon thirty (30) days' prior written notice by the Company to Executive of termination due to Disability. For purposes of this Agreement, "Disability" shall be defined as the inability of Executive to perform Executive's material duties hereunder due to a physical or mental injury, infirmity or incapacity for one hundred eighty (180) days (including weekends and holidays) in any 365-day period. Notwithstanding the foregoing, in the event that as a result of earlier absence because of mental or physical incapacity, Executive incurs a "separation from service" within the meaning of such term under Code Section 409A (as defined in Section 25(a) hereof), Executive shall on such date automatically be terminated from employment as a Disability termination.

(b) **DEATH.** Automatically on the date of death of the Executive.

(c) **CAUSE.** Subject to the notice requirements and cure periods as set forth in this Section 8(c), immediately upon written notice by the Company to Executive of a termination for Cause or upon the expiration of any applicable notice period following which the event triggering Cause has not been cured. "Cause" shall mean:

(i) Executive's substantial and repeated failure to perform in good faith Executive's duties hereunder or to follow the reasonable and legal written direction of the CEO or the Board after the Executive has received a written demand of the same from the Company which specifically sets forth the factual basis for the Company's belief that the Executive has not substantially performed and the Executive has failed to provide a sufficient explanation for, or cure such non-performance, if curable, within ten (10) business days after receiving such notice;

(ii) Executive's willful material misconduct with respect to any material aspect of the business of the Company;

(iii) Executive's conviction of or pleading of guilty or nolo contendere to, a felony or any crime involving moral turpitude;

(iv) Executive's performance of any material act of theft or fraud in connection with the performance of the Executive's duties to the Company;

(v) A material breach of this Agreement, or the Confidential Proprietary Rights and Non-Disclosure Agreement, or CIC Severance Agreement or any other restrictive covenant agreement signed by the parties or a material violation of the Company's code of conduct or other written, material, published Company policy after Executive has received written notice specifying such breach or violation, and Executive has failed provide a sufficient explanation for his conduct or to cure such breach or violation, if curable, within ten (10) business days after receiving such notice.

(d) **WITHOUT CAUSE.** Immediately upon written notice by the Company to Executive of an involuntary termination without Cause (other than for death or Disability).

(e) **GOOD REASON.** Upon thirty (30) days' prior written notice by the Executive to the Company of a termination for Good Reason. "Good Reason" shall mean the occurrence of any of the following events:

(i) Material diminution in Executive's Base Salary or Target Bonus except as provided in Section 3 of this Agreement;

(ii) Material diminution in Executive's title, authority, duties or responsibilities (other than temporarily while physically or mentally incapacitated or as required by applicable law);

(iii) Executive no longer reports to the Chief Executive Officer of the Company;

(iii) Relocation of Executive's primary work location by more than 30 miles from its then current location; or

(iv) A material breach by the Company of this Agreement, any equity award agreement, the CIC Severance Agreement or the Confidential Proprietary Rights and Non-Disclosure Agreement.

Executive shall provide the Company with a written notice detailing the specific circumstances alleged to constitute Good Reason within ninety (90) days after the first occurrence of such circumstances, and the Company shall have thirty (30) days following receipt of such notice to cure such circumstances in all material respects, provided, that, no termination for Good Reason shall occur after the 180th day following the first occurrence of a Good Reason event.

(f) **WITHOUT GOOD REASON.** Upon thirty (30) days' prior written notice by Executive to the Company of the Executive's voluntary termination of employment without Good Reason (which the Company may, in its sole discretion, make effective earlier than any notice date, except that Executive shall be treated as an active employee through the last day of the 30 day notice period).

9. CONSEQUENCES OF TERMINATION.

(a) **DEATH OR DISABILITY.** In the event that Executive's employment is terminated due to Executive's death or Disability, Executive or Executive's legal representative or estate, as the case may be, shall be entitled to (1) the "Accrued Benefits," which shall mean: (i) any earned but unpaid Base Salary through the date of termination, payable in accordance with the regular payroll practices of the Company, but no later than thirty (30) days following the date of termination; (ii) any Annual Bonus earned but unpaid with respect to the fiscal year ending on or preceding the date of termination, payable at the time such bonuses would have been paid if Executive was still employed with the Company; (iii) reimbursement for any unreimbursed business expenses incurred through the date of termination within thirty (30) days following the date of termination; (iv) any accrued but unused vacation time in accordance with Company policy; (v) any unpaid portion of the Sign-On Bonus; and (vi) all other payments, benefits or fringe benefits to which Executive shall be entitled under the terms of any applicable compensation arrangement or benefit, equity or fringe benefit plan or program or grant or this Agreement; and (2) the Restricted Stock Units and Option Award, which shall become fully vested as of the date of such termination.

(b) **TERMINATION FOR CAUSE.** If Executive's employment is terminated by the Company for Cause, the Company shall pay to Executive each of the Accrued Benefits.

(c) **TERMINATION WITHOUT CAUSE OR FOR GOOD REASON OUTSIDE OF CHANGE IN CONTROL PERIOD.** If during the Employment Term and outside of a Change in Control Period (as defined in the CIC Severance Agreement), Executive's employment by the Company is terminated (x) by the Company without Cause, or (y) by the Executive for Good Reason, the Company shall pay or provide the Executive with the following, subject to the provisions of Section 26 hereof:

(i) The Accrued Benefits;

(ii) An aggregate amount equal to the sum of (1) 12 months of Executive's then current Base Salary; and (2) Executive's Target Bonus, calculated as if 100% of performance targets were met and payable in substantially equal installments in accordance with the regular payroll policies of the Company, over a period of 12 months following the date of termination of employment, except that the first installment shall be paid on the sixtieth (60th) day following the date of termination and shall include any prior installment that would have been payable if the Release requirement set forth in Section 9 hereof were satisfied on the date of termination;

(iii) A monthly amount equal to the monthly amount of the COBRA continuation coverage premium under the Company's group medical plans as in effect from time to time less the amount of the Executive's portion of the premium as if the Executive was an active employee for a period of 12 months, except that (1) the first installment shall be paid on the sixtieth (60th) day following the date of termination and shall include any prior installment that would have been payable if the Release requirement set forth in Section 9 hereof were satisfied on the date of termination and (2) such monthly amounts shall cease on the date the Executive becomes eligible for group health coverage through a new employer;

(iv) The Restricted Stock Units granted in Section 6(a) of this Agreement shall become vested as of the date termination.

(v) Outplacement services at a level commensurate with Executive's position in accordance with the Company's practices as in effect from time to time provided that the cost of such outplacement shall not exceed $20,000; and provided, further, that such outplacement benefits shall end not later than the last day of the second calendar year that begins after the date of termination.

(d) **OTHER OBLIGATIONS.** Upon any termination of Executive's employment with the Company, Executive shall promptly resign from any position as an officer, director or fiduciary of the Company or any Company-related entity.

(e) **EXERCISE PERIOD.** In the event of a termination for death, disability, without Cause by the Company or termination for Good Reason by Executive, Executive shall have no less than 12 months from the date of termination (but in no event beyond the remaining term of such equity awards) to exercise any equity awards (i) already vested as of the date of termination; or (ii) vested in accordance with 9(c)(iv) hereof. In the event of termination for any reason other than without Cause, Good Reason or the non-renewal of the Employment Term, the time period to exercise any equity awards already vested as of the date of termination shall be as set forth in the applicable award agreements.

(f) **TERMINATION DURING A CHANGE IN CONTROL PERIOD.** In the event the Executive experiences a "Covered Termination" during the "Change in Control Period" (each term as defined in the CIC Severance Agreement) that is coincident with the Employment Term, Executive shall be entitled severance payments and benefits under the CIC Severance Agreement in accordance with its terms and conditions, without duplication of the severance payments and benefits provided under this Agreement, and Executive shall no longer be entitled to further severance payments or benefits under this Agreement. Instead, Executive shall be paid under the CIC Severance Agreement in accordance with its terms and conditions and any severance payments and benefits payable or provided under the CIC Severance Agreement shall be reduced by any severance payments and benefits previously paid or provided to Executive under this Agreement, notwithstanding anything else to the contrary in this Agreement or the CIC Severance Agreement.

10. RELEASE; NO MITIGATION. Any and all amounts payable and benefits or additional rights provided pursuant to Section 9(c) hereof, in each case, beyond the Accrued Benefits, shall be payable only if the Executive delivers to the Company and does not revoke a general release of claims in favor of the Company in the form as attached hereto as Exhibit A (the "Release"). Such Release shall be executed and delivered (and no longer subject to revocation, if applicable) within sixty (60) days following the date of termination; provided that the Company delivers to Executive such Release within seven (7) days after the date of termination. In no event shall Executive be obligated to seek other employment or take any other action by way of mitigation of the amounts payable to Executive under any of the provisions of this Agreement, nor shall the amount of any payment hereunder be reduced by any compensation earned by the Executive as a result of employment by a subsequent employer, except as provided in Section 9(c)(iv) hereof.

11. RESTRICTIVE COVENANTS.

(a) **CONFIDENTIAL PROPRIETARY RIGHTS AND NON-DISCLOSURE AGREEMENT.** Executive agrees that Executive shall be bound by the terms and conditions of the Confidential Proprietary Rights and Non-Disclosure Agreement dated as of May 23, 2018.

(b) **Non-disparagement.** Executive agrees that he will not, whether directly or indirectly, by name or innuendo, disparage or encourage or induce others to disparage the Company, the members of its Board, its officers, or any other member of the Company's senior management team (collectively, the "Company Representatives"). The Company, by and through each of its Company Representatives, shall not disparage or encourage or induce others, including the Company, to disparage Executive to third parties. For the purposes of this Agreement, the term "disparage" includes the making of false, defamatory or derogatory comments that could reasonably be expected to damage the reputation of Executive, the Company or any of the Company Representatives; provided, however, that nothing in this Agreement shall restrict communications protected as privileged under federal or state law, or any testimony or communications ordered and required by any administrative agency or court of competent jurisdiction.

(c) **Non-Interference**. During Executive's employment with the Company and for a period of one (1) year thereafter (such one (1) year period, the "Restriction Period"), Executive agrees that he/she shall not, except in the furtherance of his/her duties hereunder, directly or indirectly, individually or on behalf of any other person, firm, corporation or other entity, solicit, aid or induce any customer of the Company or any of its subsidiaries or affiliates to purchase goods or services then sold by the Company or any of its subsidiaries or affiliates from another person, firm, corporation or other entity.

(d) **Non-Solicitation**. During Executive's employment with the Company and for the Restriction Period, Executive agrees that he/she shall not, except in the furtherance of his/her duties hereunder, directly or indirectly, individually or on behalf of any other person, firm, corporation or other entity, (i) solicit, aid or induce any employee, representative or agent of the Company or any of its subsidiaries or affiliates to leave such employment or retention or to accept employment with or render services to or with any other person, firm, corporation or other entity unaffiliated with the Company or hire or retain any such employee, representative or agent, or take any action to materially assist or aid any other person, firm, corporation or other entity in identifying, hiring or soliciting any such employee, representative or agent, or (ii) interfere, or aid or induce any other person or entity in interfering, with the relationship between the Company or any of its subsidiaries or affiliates and any of their respective vendors, joint venturers, or licensors. An employee, representative or agent shall be deemed covered by (i) above while so employed or retained and for a period of six (6) months thereafter; provided however, the provisions of (i) above shall not be violated by (A) general advertising or solicitation not specifically targeted at Company-related persons or entities, (B) Executive serving as a reference, upon request, for any employee of the Company or any of its subsidiaries or affiliates, or (C) actions taken by any person or entity with which Executive is associated if Executive is not personally involved in any manner in the matter and has not identified such Company-related person or entity for soliciting or hiring.

(e) **Non-Competition Covenant**. Executive acknowledges that he/she perform services of a unique nature for the Company that are irreplaceable, and that Executive's performance of such services for a competing business will result in irreparable harm to the Company. Accordingly, during Executive's employment hereunder and for the Restriction Period, Executive agrees that he/she will not, directly or indirectly, own, manage, operate, control, be employed by (whether as an employee, consultant, independent contractor or otherwise, and whether or not for compensation) or render services to any person, firm, corporation or other entity, in whatever form, engaged in the research, development or sale of Duchenne Muscular Dystrophy treatments ("DMD"), oligonucleotide based therapies with respect to DMD, or chemistry platforms with respect to DMD that compete with Company or any of its subsidiaries or affiliates or in any other material business in which the Company or any of its subsidiaries or affiliates is engaged on the date of termination or in which they have planned, on or prior to such date, to be engaged in on or after such date, in any locale of any country in which the Company conducts business. Notwithstanding the foregoing, nothing herein shall prohibit Executive from being a passive owner of not more than one percent (1%) of the equity securities of a publicly traded corporation engaged in a business that is in competition with the Company or any of its subsidiaries or affiliates, so long as Executive has no active participation in the business of such corporation. In addition, the provisions of this Sub-Section (e) shall not be violated by Executive commencing employment with a subsidiary, division, or unit of any entity that engages in a business in competition with the Company or any of its subsidiaries or affiliates so long as Executive and such subsidiary, division or unit do not engage in a business in competition with the Company or any of its subsidiaries or affiliates.

(f) **RETURN OF COMPANY PROPERTY.** On the date of Executive's termination of employment with the Company for any reason (or at any time prior thereto at the Company's request), Executive shall return all property belonging to the Company or its affiliates (including, but not limited to, any Company-provided laptops, computers, cell phones, wireless electronic mail devices or other equipment, or documents and property belonging to the Company). Executive may retain Executive's rolodex and similar address books or a copy of his Outlook Contacts provided that such items only include contact information. To the extent that Executive is provided with a cell phone number by the Company during employment, the Company shall cooperate with Executive in transferring such cell phone number to Executive's individual name following the date of termination.

(g) **SURVIVAL OF PROVISIONS.** The obligations contained in Sections 9, 11, 12, 15, 19, 20, 21 and 22 hereof as well as those set forth in the Confidential Proprietary Rights and Non-Disclosure Agreement attached as Exhibit B (the "Confidentiality Agreement") shall survive the termination or expiration of the Employment Term and any termination of the Executive's employment with the Company and shall be fully enforceable thereafter.

(h) **Tolling**. In the event Executive violates any of the provisions of Sub-Sections (c)-(e) above, Executive acknowledges and agrees that the post-termination restrictions contained in Sub-Sections (c)-(e) shall be extended by a period of time equal to the period of such violation, it being the intention of the parties hereto that the running of the applicable post-termination restriction period shall be tolled during any period of such violation.

(i) **Court's Authority to Revise**. If any provision in this Section is found by a court of competent jurisdiction to be unenforceable or unreasonable as written, Executive and the Company hereby specifically and irrevocably authorize and request said court to revise the unenforceable or unreasonable provisions in a manner that shall result in the provision being enforceable while remaining as similar as legally possible to the purpose and intent of the original.

12. COOPERATION. Upon the receipt of reasonable notice from the Company (including outside counsel), Executive agrees that while employed by the Company (and at such times that are reasonably convenient for Executive after termination of Executive's employment with the Company), Executive will respond and provide information with regard to matters in which Executive has knowledge as a result of Executive's employment with the Company, and will assist while employed (and will provide reasonable assistance after termination of Executive's employment with the Company) to the Company, its affiliates and their respective representatives in defense of any claims that may be made against the Company or its affiliates, and will assist while employed (and will provide reasonable assistance after termination of Executive's employment with the Company) to the Company and its affiliates in the prosecution of any claims that may be made by the Company or its affiliates, to the extent that such claims may relate to the period of Executive's employment with the Company. Executive agrees that while employed by the Company, Executive will promptly inform the Company if Executive becomes aware of any lawsuit or government investigation involving any claim that may be filed or threatened against the Company or its affiliates, and that after termination of Executive's employment with the Company, Executive will promptly inform the Company if Executive (i) is served with a complaint, summons, subpoena, pleading, order or other similar document relating to the Company or its affiliates, or (ii) otherwise receives written notice of any lawsuit, government investigation or regulatory body action involving any claim that may be filed or threatened against the Company or its affiliates. Executive also agrees to promptly inform the Company (to the extent that Executive is legally permitted to do so) if Executive is asked to assist in any investigation of the Company or its affiliates (or their actions), regardless of whether a lawsuit or other proceeding has then been filed against the Company or its affiliates with respect to such investigation, and shall not do so unless legally required. Upon presentation of appropriate documentation, the Company shall pay or reimburse Executive for all reasonable out-of-pocket travel, duplicating or telephonic expenses incurred by the Executive in complying with this Section 12, including reasonable attorneys' fees, and, in the event Executive is no longer receiving any compensation or benefits under this Agreement or as a Company employee, shall pay Executive a reasonable hourly rate for any work performed at Company's request. Notwithstanding anything in this Section 12 to the contrary, Executive shall not be required to comply with the provisions of this Section 12 to the extent Executive is required by a court order, judicial process or directive of a governmental agency not to comply with all or any portion of any provision in this Section 12.

13. EQUITABLE RELIEF AND OTHER REMEDIES. Executive acknowledges and agrees that the Company's remedies at law for a breach or threatened breach of any of the provisions of Section 11 hereof would be inadequate and, in recognition of this fact, Executive agrees that, in the event of such a breach or threatened breach,

in addition to any remedies at law, the Company, without posting any bond, shall be entitled to obtain equitable relief in the form of specific performance, a temporary restraining order, a temporary or permanent injunction or any other equitable remedy which may then be available. In the event that a court of competent jurisdiction or an arbitrator has finally determined that Executive has violated Section 11 hereof, any payments made to Executive under Section 9, except for the Accrued Benefits shall immediately cease, and any payments made under Section 9 other than the Accrued Benefits to the Executive shall be immediately repaid by Executive to the Company.

14. NO ASSIGNMENTS. This Agreement is personal to each of the parties hereto. Except as provided in this Section 14 hereof, no party may assign or delegate any rights or obligations hereunder without first obtaining the written consent of the other party hereto. Subject to the terms of the CIC Severance Agreement, the Company may assign this Agreement to any successor to all or substantially all of the business and/or assets of the Company, provided that the Company shall require such successor to expressly assume and agree in writing to perform this Agreement in accordance with its terms and to the same extent that the Company would be required to perform it if no such succession had taken place. As used in this Agreement, "Company" shall mean the Company and any successor to its business and/or assets, which assumes and agrees in writing to perform the duties and obligations of the Company under this Agreement by operation of law or otherwise.

15. NOTICE. For purposes of this Agreement, notices and all other communications provided for in this Agreement shall be in writing and shall be deemed to have been duly given (a) on the date of delivery, if delivered by hand, (b) on the date of transmission, if delivered by confirmed facsimile or electronic mail, (c) on the first business day following the date of deposit, if delivered by guaranteed overnight delivery service, or (d) on the fourth business day following the date delivered or mailed by United States registered or certified mail, return receipt requested, postage prepaid, addressed as follows:

If to the Executive:

At the address (or to the facsimile number) shown
on the records of the Company

With a copy to counsel:
Hollis Gonerka Bart LLP
3 Columbus Circle, 15th Floor
New York, NY 10019

If to the Company:

Sarepta Therapeutics, Inc.
215 First St.
Cambridge, MA 02142
Attention: Ty Howton, General Counsel

or to such other address as either party may have furnished to the other in writing in accordance herewith, except that notices of change of address shall be effective only upon receipt.

16. SECTION HEADINGS; INCONSISTENCY. The section headings used in this Agreement are included solely for convenience and shall not affect, or be used in connection with, the interpretation of this Agreement. In the event of any inconsistency between the terms of this Agreement or the CIC Severance Agreement, as applicable, and any form, award, plan or policy of the Company, the terms of this Agreement or the CIC Severance Agreement, as applicable, shall govern and control.

17. SEVERABILITY. The provisions of this Agreement shall be deemed severable and the invalidity or unenforceability of any provision shall not affect the validity or enforceability of the other provisions hereof.

18. COUNTERPARTS. This Agreement may be executed in several counterparts, each of which shall be deemed to be an original but all of which together will constitute one and the same instrument.

19. ARBITRATION. Any dispute or controversy arising under or in connection with this Agreement or Executive's employment with the Company, other than injunctive relief under Section 13 hereof, shall be resolved as follows:

a) First, parties will attempt to resolve the dispute or controversy by negotiations between the parties. Either party may declare the negotiations at an impasse.

b) Second, if the negotiations do not succeed in resolving the dispute or controversy, the dispute or controversy shall be submitted to non-binding mediation before a mutually agreeable private mediator. Either party may declare the mediation at an impasse; and

c) Finally, if the dispute or controversy is not resolved through mediation, the dispute or controversy shall be resolved exclusively by arbitration, conducted before a single arbitrator in Boston, Massachusetts (applying Massachusetts law) in accordance with the National Rules for the Resolution of Employment Disputes of the American Arbitration Association then in effect. The decision of the arbitrator will be final and binding upon the parties hereto. Judgment may be entered on the arbitrator's award in any court having jurisdiction. The parties acknowledge and agree that in connection with any such arbitration and regardless of outcome, (a) each party shall pay all of its own costs and expenses, including, without limitation, its own legal fees and expenses, and (b) the arbitration costs shall be borne entirely by the Company.

20. INDEMNIFICATION. The Company hereby agrees to indemnify Executive and hold Executive harmless to the fullest extent permitted by law against and in respect of any and all actions, suits, proceedings, claims, demands, judgments, costs, expenses (including advancement of reasonable attorney's fees), losses, and damages resulting from Executive's good faith performance of Executive's duties and obligations with the Company and the Company's affiliates. These obligations shall survive the expiration of the Employment Term and the termination of Executive's employment with the Company.

21. LIABILITY INSURANCE. The Company shall cover Executive under directors' and officers' liability insurance both during and, while potential liability exists, after the term of this Agreement in the same amount and to the same extent as the Company covers its other officers and directors. These obligations shall survive the expiration of the Employment Term and the termination of Executive's employment with the Company.

22. MISCELLANEOUS. No provision of this Agreement may be modified, waived or discharged unless such waiver, modification or discharge is agreed to in writing and signed by Executive and such officer or director of the Company as may be designated by the Board. No waiver by either party hereto at any time of any breach by the other party hereto of, or compliance with, any condition or provision of this Agreement to be performed by such other party shall be deemed a waiver of similar or dissimilar provisions or conditions at the same or at any prior or subsequent time. This Agreement, together with all exhibits hereto, the CIC Severance Agreement, the equity and incentive award agreements, set forth the entire agreement of the parties hereto in respect of the subject matter contained herein and supersede any and all prior agreements or understandings between the Executive and the Company with respect to the subject matter hereof. No agreements or representations, oral or otherwise, express or implied, with respect to the subject matter hereof have been made by either party which are not expressly set forth in this Agreement. The validity, interpretation, construction and performance of this Agreement shall be governed by the laws of the Commonwealth of Massachusetts without regard to the choice of law principles thereof.

23. REPRESENTATIONS. Executive represents and warrants to the Company that (a) Executive has the legal right to enter into this Agreement and to perform all of the obligations on Executive's part to be performed hereunder in accordance with its terms, and (b) Executive is not a party to any agreement or understanding, written or oral, and is not subject to any restriction, which, in either case, could prevent the Executive from entering into this Agreement or performing all of Executive's duties and obligations hereunder. In addition, the Executive acknowledges that the Executive is aware of Section 304 (Forfeiture of Certain Bonuses and Profits) of the Sarbanes-Oxley Act of 2002 and the right of the Company to be reimbursed for certain payments to the Executive in compliance with any policy Company may adopt in connection therewith.

24. LEGAL FEES. Within thirty (30) days upon presentation of appropriate documentation, the Company shall pay all reasonable and documented legal fees and related expenses incurred in connection with the drafting, negotiation and execution of this Agreement and associated agreements referenced herein, up to a maximum of $20,000.

25. TAX WITHHOLDING. The Company may withhold from any and all amounts payable under this Agreement such federal, state and local taxes as may be required to be withheld pursuant to any applicable law or regulation.

26. CODE SECTION 409A COMPLIANCE.

(a) The intent of the parties is that payments and benefits under this Agreement comply with, or be exempt from, Internal Revenue Code Section 409A and the regulations and guidance promulgated thereunder (collectively "Code Section 409A") and, accordingly, to the maximum extent permitted, this Agreement shall be interpreted to be in compliance therewith. If the Executive notifies the Company (with specificity as to the reason therefor) that the Executive believes that any provision of this Agreement (or of any award of compensation, including equity compensation or benefits) would cause the Executive to incur any additional tax or interest under Code Section 409A and the Company concurs with such belief or the Company (without any obligation whatsoever to do so) independently makes such determination, the Company shall, after consulting with the Executive, reform such provision to try to comply with Code Section 409A through good faith modifications to the minimum extent reasonably appropriate to conform with Code Section 409A. To the extent that any provision hereof is modified in order to comply with Code Section 409A, such modification shall be made in good faith and shall, to the maximum extent reasonably possible, maintain the original intent and economic benefit to the Executive and the Company of the applicable provision without violating the provisions of Code Section 409A.

(b) A termination of employment shall not be deemed to have occurred for purposes of any provision of this Agreement providing for the payment of any amounts or benefits upon or following a termination of employment that are considered "nonqualified deferred compensation" under Code Section 409A unless such termination is also a "separation from service" within the meaning of Code Section 409A and, for purposes of any such provision of this Agreement, references to a "termination," "termination of employment" or like terms shall mean "separation from service." If the Executive is deemed on the date of termination to be a "specified employee" within the meaning of that term under Code Section 409A(a)(2)(B), then

with regard to any payment that is considered non-qualified deferred compensation under Code Section 409A (and not otherwise exempt under Code Section 409A) payable on account of a "separation from service," such payment or benefit shall be made or provided at the date which is the earlier of (i) the expiration of the six (6)-month period measured from the date of such "separation from service" of the Executive, and (ii) the date of the Executive's death (the "Delay Period"). Upon the expiration of the Delay Period, all payments and benefits delayed pursuant to this Section 25 (whether they would have otherwise been payable in a single sum or in installments in the absence of such delay) shall be paid or reimbursed to the Executive in a lump sum without interest, and any remaining payments and benefits due under this Agreement shall be paid or provided in accordance with the normal payment dates specified for them herein.

(c) With regard to any provision herein that provides for reimbursement of costs and expenses or in-kind benefits, except as permitted by Code Section 409A, (i) the right to reimbursement or in-kind benefits shall not be subject to liquidation or exchange for another benefit, (ii) the amount of expenses eligible for reimbursement, or in-kind benefits, provided during any taxable year shall not affect the expenses eligible for reimbursement, or in-kind benefits to be provided, in any other taxable year, provided that the foregoing clause (ii) shall not be violated without regard to expenses reimbursed under any arrangement covered by Internal Revenue Code Section 105(b) solely because such expenses are subject to a limit related to the period the arrangement is in effect and (iii) such payments shall be made on or before the last day of Executive's taxable year following the taxable year in which the expense occurred.

(d) For purposes of Code Section 409A, the Executive's right to receive any installment payments pursuant to this Agreement shall be treated as a right to receive a series of separate and distinct payments. In no event may the Executive, directly or indirectly, designate the calendar year of any payment to be made under this Agreement that is considered nonqualified deferred compensation. In no event shall the timing of Executive's execution of the Release, directly or indirectly, result in the Executive designating the calendar year of payment, and if a payment that is subject to execution of the Release could be made in more than one taxable year, payment shall be made in the later taxable year.

27. CLAWBACK. Notwithstanding anything herein to the contrary, Executive agrees and acknowledges that his cash and non-cash incentive compensation (whether provided under this Agreement or otherwise), excluding the Restricted Stock Units, shall be subject to the terms and conditions of the Company's Incentive Compensation Recoupment Policy approved by the Company in April 2016, as amended from time to time. Notwithstanding the foregoing, Executive agrees that incentive compensation, as defined under the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010 and such regulations as are promulgated thereunder from time to time ("Dodd-Frank"), payable to the Executive under the Company's bonus plans, this Agreement or any other plan, arrangement or program established or maintained by the Company shall be subject to any clawback policy adopted or implemented by the Company in compliance with Dodd-Frank, or in respect of any other applicable law or regulation. Further, any shares acquired pursuant to the vesting of any equity award other than the Restricted Stock Units or pursuant to the exercise of the Performance Option Award (or any other option) shall be subject to clawback by the Company as a result of any act or omission that involves the Executive's fraud or any act or omission of the Executive that constitutes Cause, as defined in Section 8(c) hereof.

[signature page to follow]

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date first written above.

SAREPTA THERAPEUTICS, INC.

By: **/s/ Douglas S. Ingram**
Douglas S. Ingram

Its: Chief Executive Officer

Date: May 23, 2018

EXECUTIVE

By: **/s/ Dr. Gilmore N. O'Neill**
Dr. Gilmore N. O'Neill

Date: May 23, 2018

EXHIBIT A

GENERAL RELEASE

GENERAL RELEASE (the "Release"), by **Dr. Gilmore O'Neill** (the "Executive") in favor of **Sarepta Therapeutics, Inc.** (the "Company") and the Company Releasees (as hereinafter defined), dated as of May 23, 2018.

Capitalized terms used herein but not specifically defined shall have the meanings set forth in the Employment Agreement between Executive and the Company, dated as of May 23, 2018 (the "Employment Agreement").

WHEREAS, in connection with the termination of Executive's employment, the Company has agreed to provide Executive with the payments and benefits set forth in the Employment Agreement, subject to the terms and conditions set forth therein.[1]

NOW, THEREFORE, in consideration of the covenants and agreements hereinafter set forth, the parties agree as follows:

1. General Release. Executive, for Executive and for Executive's heirs, executors, administrators, successors and assigns (referred to collectively as "Releasors") hereby irrevocably and unconditionally, and knowingly and voluntarily, waives, terminates, cancels, releases and discharges forever the Company, and its subsidiaries, affiliates and related entities, and any and all of their respective predecessors, successors, assigns and employee benefit plans, together with each of their respective owners, assigns, agents, directors, general and limited partners, shareholders, directors, officers, employees, attorneys, advisors, trustees, fiduciaries, administrators, agents or representatives, and any of their predecessors and successors and each of their estates, heirs and assigns (collectively, the "Company Releasees") from any and all charges, allegations, complaints, claims, liabilities, obligations, promises, agreements, causes of action, rights, costs, losses, debts and expenses of any nature whatsoever, including those arising from or related to the Executive's Change in Control and Severance Agreement, dated May 23, 2018, known or unknown, suspected or unsuspected (collectively, "Claims") which Executive or the Releasors ever had, now have, may have, or hereafter can, will or may have (either directly, indirectly, derivatively or in any other representative capacity) by reason of any matter, fact or cause whatsoever against the Company or any of the other Company Releasees: (a) from the beginning of time to the date upon which Executive signs this Agreement, (b) arising out of, or relating to, Executive's employment with the Company and/or the termination of Executive's employment; or (c) arising out of or related to any agreement or arrangement between Executive and/or any Company Releasees. This Release includes, without limitation, all claims for attorneys' fees and punitive or consequential damages and all claims arising under any federal, state and/or local labor, employment, whistleblower and/or anti-discrimination laws and/or regulations, including, without limitation, the Age Discrimination in Employment Act of 1967 ("ADEA"), Title VII of the Civil Rights Act of 1964, the Employee Retirement Income Security Act, the Americans with Disabilities Act, the Family and Medical Leave Act, the Civil Rights Act of 1991, the Equal Pay Act, the Immigration and Reform Control Act, the Uniform Services Employment and Re-Employment Act, the Rehabilitation Act of 1973, Executive Order 11246, the Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the Worker Adjustment Retraining and Notification Act and the Family Medical Leave Act, the Massachusetts Fair Employment Practices Statute (M.G.L. c. 151B § 1, *et seq.*), the Massachusetts Equal Rights Act (M.G.L. c. 93, §102), the Massachusetts Civil Rights Act (M.G.L. c. 12, §§ 11H & 11I), the Massachusetts Privacy Statute (M.G.L. c. 214, § 1B), the Massachusetts Sexual Harassment Statute (M.G.L. c. 214, § 1C), the Massachusetts Wage Act (M.G.L. c. 149 § 148, *et seq.*), the Massachusetts Minimum Fair Wages Act (M.G.L. c. 151 § 1, *et seq.*), the Massachusetts Equal Pay Act (M.G.L. c. 149, § 105A), and any similar Massachusetts or other state or federal statute, including all amendments to any of the aforementioned acts or under any common law or equitable theory including, but not limited to, tort, breach of contract, fraud, fraudulent

[1] The specifics of the actual payments will be added consistent with the Employment Agreement.

inducement, promissory estoppel or defamation, and violations of any other federal, state, or municipal fair employment statutes or laws, including, without limitation, violations of any other law, rule, regulation, or ordinance pertaining to employment, wages, compensation, hours worked, or any other matters related in any way to the foregoing; provided, however, that nothing in this Release shall release or impair any rights that cannot be waived under applicable law.

2. Surviving Claims. Notwithstanding anything herein to the contrary, this Release shall not:

a. limit or prohibit in any way Executive's (or Executive's beneficiaries' or legal representatives') rights to bring an action to enforce the terms of the Employment Agreement or this Release, or for the Company's reimbursement of business expenses incurred by Executive but unpaid in accordance with the Company's expenses reimbursement policies;

b. release any claim for employee benefits under plans covered by the Employee Retirement Income Security Act of 1974, as amended, to the extent that such claims may not lawfully be waived, or for any payments or benefits under any benefit plans of the Company and its affiliates in which Executive was a participant as of the date of termination of Executive's employment that have accrued or vested in accordance with and pursuant to the terms of those plans;

c. release any claims for indemnification (i) in accordance with applicable laws or the corporate governance documents of the Company or its affiliates in accordance with their terms as in effect from time to time, (ii) pursuant to any applicable directors and officers insurance policy with respect to any liability incurred by Executive as an officer or director of the Company or its affiliates in accordance with the terms thereof or (iii) pursuant to the terms of Sections 19 and 20 of the Employment Agreement.

3. Executive Representations. Executive represents and warrants that the Releasors have not filed any civil action, suit, arbitration, administrative charge, complaint, lawsuit or legal proceeding against any Company Releasee nor has any Releasor assigned, pledged, or hypothecated, as of the Effective Date, Executive's claim to any person and no other person has an interest in the Claims that Executive is releasing.

4. Acknowledgements by Executive. Executive acknowledges and agrees that Executive has read this Release in its entirety and that this Release is a general release of all known and unknown rights and Claims, including, without limitation, of rights and Claims arising under ADEA. This Release specifically includes a waiver and release of Claims that Executive has or may have regarding payments or amounts covered by the Massachusetts Wage Act or the Massachusetts Minimum Fair Wages Act (including, for instance, hourly wages, salary, overtime, minimum wages, commissions, vacation pay, holiday pay, sick leave pay, dismissal pay, bonus pay or severance pay), as well as Claims for retaliation under the Massachusetts Wage Act or the Massachusetts Minimum Fair Wages Act. Executive further acknowledges and agrees that:

a. this Release does not release, waive or discharge any rights or claims that may arise for actions or omissions after the date of this Release;

b. Executive is entering into this Release and releasing, waiving and discharging rights or claims only in exchange for consideration which Executive is not already entitled to receive;

c. Executive has been advised, and is being advised by this Release, to consult with an attorney before executing this Release, and Executive has consulted with counsel of Executive's choice concerning the terms and conditions of this Release;

d. Executive has been advised, and is being advised by this Release, that Executive has forty-five (45) days within which to consider this Release, and Executive hereby acknowledges that in the event that Executive executes this Release prior to the expiration of the 45-day period, Executive waives the balance of said period and acknowledges that Executive's waiver of such period is knowing, voluntary and has not been induced by the Company or any Company Releasee through fraud, misrepresentation, or threat; and

e. Executive is aware that this Release shall become null and void if Executive revokes Executive's agreement to this Release within seven (7) days following the date of execution of this Release. Executive may revoke this Release at any time during such seven-day period by delivering (or causing to be delivered) to the General Counsel of the Company at 215 First Street, Cambridge, MA 02142, written notice of Executive's revocation of this Release no later than 5:00 p.m. Eastern Time on the seventh (7th) full day following the date of execution of this Release (the "Effective Date").

5. Additional Agreements. Nothing in this Agreement shall prohibit Executive from filing a charge with, providing information to or cooperating with any governmental agency and in connection therewith obtaining a reward or bounty, but Executive agrees that should any person or entity file or cause to be filed any civil action, suit, arbitration, or other legal proceeding seeking equitable or monetary relief concerning any claim released by Executive herein, neither the Executive nor any Releasor shall seek or accept any such damages or relief from or as the result of such civil action, suit, arbitration, or other legal proceeding filed by Executive or any action or proceeding brought by another person, entity or governmental agency. In addition, nothing in this Release shall be construed to prohibit Executive from (a) reporting or disclosing information under the terms of the Company's *Reporting Suspected Violations of Law Policy* or (b) reporting possible violations of federal and/or state law or regulations, including any possible securities laws violations, to any governmental agency or entity, including the U.S. Department of Justice, the U.S. Securities and Exchange Commission, the U.S. Congress, or any agency Inspector General; making any other disclosures that are protected under the whistleblower provisions of federal and/or state law or regulations; otherwise fully participating in any federal and/or state whistleblower programs, including any such programs managed by the U.S. Securities and Exchange Commission or the Occupational Safety and Health Administration; or receiving individual monetary awards or other individual relief by virtue of participating in any such federal and/or state whistleblower programs (it being understood that prior authorization of the Company is not required to make any such reports or disclosures, and the Executive is not required to notify the Company that he or she has made such reports or disclosures). Additionally, the Executive acknowledges and understands that under the Federal Defend Trade Secrets Act of 2016, the Executive shall 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: (i) (A) in confidence to a federal, state or local government official, either directly or indirectly, or to an attorney; and (B) solely for the purpose of reporting or investigating a suspected violation of law; (ii) to the Executive's attorney in relation to a lawsuit for retaliation against the Executive for reporting a suspected violation of law; or (iii) in a complaint or other document filed in a lawsuit or other proceeding, if such filing is made under seal

6. Amendment. No provision of this Release may be modified, changed, waived or discharged unless such waiver, modification, change or discharge is agreed to in writing and signed by the Company and Executive.

IN WITNESS WHEREOF, Executive has signed this Release on the date set forth below.

EXECUTIVE

By:

Dr. Gilmore N. O'Neill

EXHIBIT B

CONFIDENTIAL PROPRIETARY RIGHTS AND NON-DISCLOSURE AGREEMENT

This Confidential Proprietary Rights and Non-Disclosure Agreement (this "Agreement") by and between Sarepta Therapeutics, Inc., a Delaware corporation, on behalf of itself, its subsidiaries and other corporate affiliates ("Sarepta") and Dr. Gilmore N. O'Neill ("Employee") (each, a "Party" and, collectively, the "Parties") is effective as of June 7, 2018 (the "Effective Date"), subject to and conditioned upon the Employee's commencement of employment with the Company on such date.

RECITALS

A. Employee will be engaged as an employee to provide services to Sarepta (the "Services") as an at-will employee.

B. Employee will have access to certain material, non-public information about Sarepta.

C. As a condition precedent to providing such information to the Employee in connection with the Services and Employee's employment, the Parties have agreed to enter into this Agreement.

NOW, THEREFORE, as a condition of employment and continued employment with Sarepta, and in consideration of the mutual covenants expressed herein, the receipt and sufficiency of which are acknowledged, the Parties, intending to be legally bound, agree as follows.

AGREEMENT

1.Definitions. For the purposes of this Agreement:

1.1 "Affiliate" of a Party means any entity that a Party directly or indirectly controls, or is controlled by, including but not limited to employees, agents, and entities.

1.2 "Representative" means, with respect to either Party, such Party's members, managers, partners, Affiliates, attorneys, advisors, potential lenders, potential co-investors, directors, officers, employees, agents, representatives, or family members.

1.3 "Confidential Information" means any business, marketing, technical, or other information in tangible or intangible form disclosed by Sarepta to Employee that, at the time of disclosure, is designated as confidential (or like designation), is disclosed in circumstances of confidence, or would be understood by the Parties (or their Affiliates and Representatives), exercising reasonable business judgment, to be confidential. This includes information that is conceived, compiled, developed, discovered or received by, or made available to Employee during his/her Employment, and whether or not while engaged in performing work for Sarepta. Confidential Information includes information, both written and oral, relating to Inventions, trade secrets and other proprietary information, technical data, products, services, unpublished financial information or projections, business, marketing and strategic plans, future service and product development plans, legal affairs, suppliers, customers, prospects, opportunities, contracts or assets of Sarepta. It specifically includes but is not limited to Sarepta business plans, product concepts, technical know-how, methods of and other information relating to operations, development strategies, distribution arrangements, financial data, marketing plans, and business practices, policies, or objectives. Confidential Information also includes any information which has been made available to Sarepta by or with respect to third parties and which Sarepta is obligated to keep confidential.

1.4 "Invention" means any product, device, technique, know-how, computer program, algorithm, method, process, procedure, improvement, discovery or invention, whether or not patentable or copyrightable and whether or not reduced to practice, that (a) is within the scope of Sarepta's business, research or investigations or results from or is suggested by any work performed by Employee for Sarepta and (b) is created, conceived, reduced to practice, developed, discovered, invented or made by Employee during the Term, whether solely or jointly with others, and whether or not while engaged in performing work for Sarepta.

1.5 "Material" means any product, prototype, model, document, diskette, tape, picture, design, recording, writing or other tangible item which contains or manifests, whether in printed, handwritten, coded, magnetic or other form, any Confidential Information, Invention or Proprietary Right.

1.6 "Proprietary Right" means any patent, copyright, trade secret, trademark, trade name, service mark, maskwork or other protected intellectual property right in any Confidential Information, Invention or Material.

1.7 "Work Product" means any information, created by Employee, Sarepta, and/or jointly by Employee and Sarepta during Employee's employment with Sarepta in connection with Sarepta's research, development and commercialization of drugs and related products, including but not limited to, data, reports, analysis, summaries, formulae, ideas, research, developments, inventions (patentable or not), processes, designs, drawings, works, clinical data and analysis, biological materials, chemical formulas, trade secrets, concepts, know-how, improvements, techniques, products, and any and all results of the research and development process.

1.8 "Term" means the term of Employee's employment or independent contracting relationship with Sarepta, whether on a full-time or part-time basis, as well as the period preceding execution of this Agreement, retroactive to the first date of Employee's employment, subject to section 7.1 below.

2.Disclosure, Use Restrictions and Proprietary Rights.

2.1 Disclosure and Use.

Except as expressly provided in this Agreement, during the Term and thereafter, Employee shall retain all Confidential Information in confidence and shall not, unless Employee obtains Sarepta's prior written consent (which may be withheld at Sarepta's sole discretion), directly or indirectly, disclose, reveal, divulge, publish or otherwise make known any of the Confidential Information for any reason or purpose whatsoever, except as required for performance of Employee's work for Sarepta and then only on a need to know basis used only in accordance with this Agreement. Employee shall take all steps necessary to safeguard and protect the Confidential Information from unauthorized access, use or disclosure by or to others, including but not limited to, maintaining appropriate security measures. The obligations of confidence set forth in this Agreement shall extend to any of Employee's Representatives that may receive Confidential Information and Employee shall be responsible for any breach of this Agreement by his or her Representatives.

In accordance with Section 2.3 below, Employee shall notify Sarepta immediately upon discovery of any unauthorized use or disclosure of Confidential Information or any other breach of this Agreement by Employee or his or her Representatives, and will cooperate with Sarepta to assist Sarepta to regain possession of the Confidential Information and prevent its further unauthorized use or disclosure.

Exemptions. Employee shall not be bound by the obligations restricting disclosure and use set forth in this Agreement with respect to Confidential Information, or any part thereof, which: (i) was known by Employee prior to his/her employment with Sarepta; (ii) prior to its disclosure or use by Employee, was already lawfully in the public domain or publicly available other than through a breach of this Agreement; (iii) was disclosed to Employee by a third party, provided

such third party is not in breach of any confidentiality obligation in respect of such information; (iv) is independently developed by Employee, where the burden is on Employee to prove independent development; or (v) is disclosed when such disclosure is compelled pursuant to legal, judicial or administrative proceedings, or otherwise required by law, subject to Employee giving reasonable prior notice to Sarepta to allow Sarepta to seek protective court orders. The foregoing exemptions shall extend to any Representatives that receive or have received Confidential Information.

For the avoidance of doubt, this Agreement does not prohibit or restrict Employee from lawfully (A) communicating or cooperating with, providing relevant information to, or otherwise assisting in an investigation by any governmental or regulatory body or official(s) or self-regulatory organization regarding a possible violation of any federal law relating to fraud or any rule or regulation of the Securities and Exchange Commission; (B) filing an administrative complaint with the Equal Employment Opportunity Commission, U.S. Department of Labor, National Labor Relations Board, or other federal, state or local agency responsible for administering fair employment, wage-hour, labor and other employment laws and regulations; (C) cooperating in an investigation, or responding to an inquiry from any such agency; or (D) testifying, participating in, or otherwise assisting in an action or proceeding relating to a possible violation of any such law, rule or regulation; provided, however, that Employee agrees that, to the maximum extent permitted by law, the Employee waives any claim for individual monetary relief in connection with any such action, investigation or proceeding.

2.2 Proprietary Rights. During the Term, Employee (including his or her Representatives) shall not acquire any rights, express or implied, in the Confidential Information of Sarepta (including its Affiliates), except for the limited use specified in this Agreement. The Confidential Information, including all right, title and interest therein, remains the sole and exclusive property of Sarepta (and its Affiliates).

2.3 Compulsory Disclosure. If Employee is legally compelled to disclose any of the Confidential Information, Employee shall promptly provide written notice to Sarepta to enable Sarepta (at its sole cost and expense) to seek a protective order or other appropriate remedy to avoid public or third-party disclosure of its Confidential Information. If such protective order or other remedy is not obtained, Employee shall furnish only so much of the Confidential Information that it is legally compelled to disclose, and shall exercise its commercially reasonable efforts to obtain reliable assurance that confidential treatment will be accorded the Confidential Information. Employee shall cooperate with and assist Sarepta, at Sarepta's expense, in seeking any protective order or other relief requested pursuant to this Section 2.3.

2.4 DTSA Notice. Nothing in this Agreement is intended to interfere with or discourage Employee from communicating with government agencies about possible violations of law or otherwise providing information to government agencies or participating in government agency investigations or proceedings. Employee is not required to notify Sarepta of any such communications; provided however, that nothing herein authorizes the disclosure of information Employee obtained through a communication that was subject to the attorney-client privilege. Further, Employee is hereby advised that in accordance with the Defend Trade Secrets Act of 2016 that Employee will not be held criminally or civilly liable under any federal or state trade secret law for the disclosure of a trade secret that: (a) is made (i) in confidence to a federal, state, or local government official, either directly or indirectly, or to an attorney; and (ii) solely for the purpose of reporting or investigating a suspected violation of law; or (b) is made in a complaint or other document that is filed under seal in a lawsuit or other proceeding.

Employee is further notified that if Employee files a lawsuit for retaliation by Sarepta for reporting a suspected violation of law, Employee may disclose Sarepta's trade secrets to Employee's attorney and use the trade secret information in the court proceeding only if Employee: (a) files any document containing the trade secret under seal; and (b) does not disclose the trade secret, except pursuant to court order.

3.Ownership of Confidential Information, Inventions, Materials, Work Product and Proprietary Rights, Non-Solicitation.

3.1 Sarepta will be the exclusive owner of all Confidential Information, Inventions, Materials, Work Product and Proprietary Rights. To the extent applicable, all Materials will constitute "works for hire" under applicable copyright laws. Employee will not at any time use Sarepta's name, trademarks, trade names or other Proprietary Rights in any advertising or publicity without Sarepta's consent.

3.2 Employee hereby assigns and transfers, and agrees to assign and transfer, to Sarepta all rights and ownership that Employee has or will have in Confidential Information, Inventions, Materials, Work Product and Proprietary Rights, subject to the limitations set forth in Section 3.5 and the Notice in Section 3.6 below. Further, Employee waives any moral rights that Employee may have in any Confidential Information, Inventions, Materials, Work Product and Proprietary Rights. Employee will take such action (including signature and assistance in preparation of documents or the giving of testimony) as may be requested by Sarepta to evidence, transfer, vest or confirm Sarepta's rights and ownership in Confidential Information, Inventions, Materials, Work Product and Proprietary Rights. Employee agrees to keep and maintain adequate and current written records of all Inventions and Proprietary Rights during the Term. The records will be in the form of notes, sketches, drawings, and any other format that may be specified by Sarepta. The records will be available to and remain the sole property of Sarepta at all times. Employee will not contest the validity of any Proprietary Right, or aid or encourage any third party to contest the validity of any Proprietary Right of Sarepta.

3.2.1 If Sarepta is unable for any reason to secure Employee's signature to fulfill the intent of the foregoing paragraph or to apply for or to pursue any application for any United States or foreign patents or copyright registrations covering Inventions assigned to Sarepta above, then Employee irrevocably appoints Sarepta and its authorized agents as Employee's agent and attorney in fact, to transfer, vest or confirm Sarepta's rights and to execute and file any such applications and to do all other lawful acts to further the prosecution and issuance of letters patent or copyright registrations with the same legal force as if done by Employee.

3.3 Except as required for performance of Employee's work for Sarepta or as authorized in writing by Sarepta, Employee will not (a) use, disclose, sell, publish or distribute any Confidential Information, Inventions, Materials, Work Product or Proprietary Rights or (b) remove any Materials from Sarepta's premises. Employee shall maintain at his or her work station and/or any other place under his or her control only such Confidential Information, Inventions, Materials, Work Product and Proprietary Rights as Employee has a current "need to know," and will return it when that need no longer exists. Employee shall not make copies of or otherwise reproduce Sarepta's Confidential Information, Inventions, Materials or Proprietary Rights, unless there is a legitimate business need of Sarepta for reproduction.

3.4 Employee will promptly disclose to Sarepta all Confidential Information, Inventions, Materials or Proprietary Rights, as well as any business opportunity which comes to Employee's attention during the Term and which relates to Sarepta's business or which arises as a result of Employee's employment with Sarepta. Employee will not take advantage of or divert any such opportunity for the benefit of Employee or any other person either during or after the Term without the prior written consent of Sarepta.

3.5 Exhibit A is a list describing inventions, original works of authorship, developments, improvements, and trade secrets which were made by Employee prior to the Term (collectively referred to as "Prior Inventions"), which belong to Employee, which relate to Sarepta's current or proposed business, products or research and development, and which are not assigned to Sarepta; or, if no such list is attached, Employee represents that there are no such Prior Inventions. If,

during the Term, Employee incorporates or allows Sarepta to incorporate into a Sarepta product, process or machine a Prior Invention owned by Employee or in which Employee has an interest, Sarepta is granted and shall have a nonexclusive, royalty-free, irrevocable, perpetual, worldwide license to make, have made, modify, use, offer to sell, and sell such Prior Invention as part of or in connection with such product, process or machine. If, during the Term, Employee incorporates or allows Sarepta to incorporate into a Sarepta product, process or machine a Prior Invention not listed in Exhibit A (Non-Listed Prior Invention), Employee shall provide to Sarepta within thirty (30) days written notice of such Non-Listed Prior Invention and written documentation proving Employee's rights to or ownership interest in that Non-Listed Prior Invention. Employee agrees that failure to timely provide such written notice and documentation bars Employee from asserting against Sarepta any rights to or ownership interest in the Non-Listed Prior Invention.

3.6 NOTICE: Notwithstanding any other provision of this Agreement to the contrary, this Agreement does not obligate Employee to assign or offer to assign to Sarepta any of Employee's rights in an invention for which no equipment, supplies, facilities or trade secret information of Sarepta was used and which was developed entirely on Employee's own time, unless (a) the invention relates (i) directly to the business of Sarepta or (ii) to Sarepta's actual or demonstrably anticipated research or development, or (b) the invention results from any work performed by Employee for Sarepta.

3.7 Non-Solicitation. Employee acknowledges and agrees that Sarepta has invested substantial time, money and resources in the development of its Confidential Information and the development and retention of its customers, clients, collaborators, and employees. Employee further acknowledges that during the course of his/her employment, he/she may be introduced to customers, clients, and collaborators of Sarepta, and agrees that any "goodwill" associated with any customer, client, or collaborator belongs exclusively to Sarepta. In recognition of the foregoing, Employee specifically acknowledges and agrees that while he/she is employed by Sarepta and for a period of one (1) year after termination of such employment (for any reason, whether voluntary or involuntary) Employee will not directly or indirectly in any position or capacity engage in the following activities for himself/herself or for any other person, business, corporation, partnership or other entity:

call upon, solicit, divert, or accept, or attempt to solicit or divert any of Sarepta's business or prospective business from any of Sarepta's customers, clients, collaborators, or prospective customers, clients or collaborators with whom Employee had contact or whose dealings with Sarepta Employee coordinated or supervised or about whom Employee obtained Confidential Information, at any time during the two (2) year period prior to the termination of Employee's employment, unless Employee obtains prior written consent of Sarepta; or

request, solicit, induce, hire (or attempt or assist in doing any of these actions) any employee or other person (including consultants) who may have performed work or services for Sarepta within one (1) year prior to the termination of Employee's employment with Sarepta to perform work or services for any person or entity other than Sarepta.

3.7.1 EMPLOYEE ACKNOWLEDGES THAT THESE RESTRICTIONS SHALL APPLY AND BE BINDING REGARDLESS OF CHANGES IN EMPLOYEE'S POSITION, DUTIES, GEOGRAPHIC LOCATION, RESPONSIBILITIES OR COMPENSATION DURING HIS/HER EMPLOYMENT.

3.7.2 If it is determined by a court of competent jurisdiction in any state that any restriction in this Section 3.7 is excessive in duration or scope or is unreasonable or unenforceable under the laws of that state, it is the intention of the Parties that such restriction may be modified or amended by the court to render it enforceable to the maximum extent permitted by the law of that state.

4.Remedies. Employee acknowledges and agrees that the provisions of this Agreement are of a special and unique nature, the loss of which cannot be accurately compensated for in damages by an action at law, and that the breach or threatened breach of this Agreement by the Employee or any of his or her Representatives would cause Sarepta and its Affiliates irreparable harm and that money damages would not be an adequate remedy. Employee agrees on behalf of him or herself and his or her Representatives that Sarepta (and its Affiliates) shall be entitled to equitable relief, including, without limitation, an injunction or injunctions (without the requirement of posting a bond, other security or any similar requirement or proving any actual damages), to prevent breaches or threatened breaches of this Agreement by Employee or any of his or her Representatives and to specifically enforce the terms and provisions of this Agreement, this being in addition to any other remedy to which Sarepta (or its Affiliates) may be entitled at law or in equity.

5.Indemnification. Employee shall indemnify and defend Sarepta and its Representatives and each of their respective directors, officers, employees, managers, members, partners, shareholders, agents and affiliates (collectively, the "Indemnified Persons") against and hold each Indemnified Person harmless from any and all liabilities, obligations, losses, damages, costs, expenses, claims, penalties, lawsuits, proceedings, actions, judgments, disbursements of any kind or nature whatsoever, interest, fines, settlements and reasonable attorneys' fees and expenses that the Indemnified Persons may incur, suffer, sustain or become subject to arising out of, relating to, or due to the breach of this Agreement by Employee or any of his or her Representatives. The provisions of this Section 5 shall survive indefinitely any termination of this Agreement, the completion or the termination of Employee's employment.

6.Securities Laws. Employee hereby acknowledges that Sarepta is a publicly traded company. Employee hereby acknowledges that Employee is aware that federal and state securities laws prohibit any person who has received material, non-public information (information about Sarepta or its business that is not generally available to the public) concerning Sarepta, including, without limitation, the matters that are the subject of this Agreement, from purchasing or selling securities of Sarepta while in possession of such non-public information, and from communicating that information to any other person who may purchase or sell securities of Sarepta or otherwise violate such laws. Employee specifically acknowledges these obligations and agrees to be bound by them, including, without limitation, Sarepta's insider trading policies in existence as of the Effective Date and as may be adopted or changed in the future.

7.Term of Confidentiality Obligation.

7.1 Term. The confidentiality obligations set forth in this Agreement shall continue with regard to an item of information as long as that information continues to meet the definition of "Confidential Information" and is not exempt under Section 2.1(c).

7.2 Return of Confidential Information. At any time upon written request by Sarepta, Employee shall return or destroy all documents or other materials embodying Confidential Information, shall retain no copies thereof, and shall certify in writing that such destruction or return has been accomplished. The confidentiality obligations set forth in this Agreement shall survive any termination of the Agreement.

8.Further Obligations.

8.1 During the Term, Employee will not, directly or indirectly, engage in, be employed by, perform services for or otherwise participate in any competing business or any other activity which conflicts with the commercial interests of Sarepta.

8.2 Employee's execution, delivery and performance of this Agreement and the performance of Employee's other obligations and duties to Sarepta will not cause any breach, default or violation of any other employment, nondisclosure, confidentiality, consulting or other agreement to which Employee is a party or by which Employee may be bound. Attached as Exhibit B is a list of all prior agreements now in effect under which Employee has agreed to keep information confidential or not to compete or solicit employees of any employer or person.

8.3 Employee agrees he or she will not use in performance of Employee's work for Sarepta or disclose to Sarepta any trade secret, confidential or proprietary information of any prior employer or other person if and to the extent that such use or disclosure may cause a breach, default or violation of any obligation or duty that Employee owes to such other person (e.g., under any agreement or applicable law). Employee warrants that Employee's compliance with this paragraph will not prohibit, restrict or impair the performance of Employee's work, obligations and duties to Sarepta.

9.Termination of Relationship.

9.1 To the extent permissible under applicable law, Employee hereby authorizes and specifically agrees to allow Sarepta to deduct from his or her wages the value of any Sarepta property (including equipment, goods, or other items provided to Employee by Sarepta during Employee's employment) which he or she fails to return when requested to do so by Sarepta, provided that such deduction (a) does not exceed the cost of the item, (b) does not reduce Employee's salary below the statutory minimum applicable to exempt employees, (c) is not made for normal wear and tear on or nonwillful loss or breakage of the provided item(s), and (d) is accompanied with a list of all items for which deductions are being made. Employee also agrees that if Sarepta loans him or her any money or advances Employee paid leave before it is earned or accrued, that Sarepta can deduct from Employee's wages the value of the balance of the unpaid loan or unaccrued paid leave.

9.2 Employee agrees that at the end of the Term, Employee will deliver to Sarepta (and will not keep in his or her possession, re-create or deliver to anyone else) any and all Materials and other property belonging to Sarepta, its successors or assigns.

9.3 Employee agrees that after the Term, Sarepta may disclose this Agreement to his or her new employer or another person to notify them of Employee's rights and obligations under this Agreement.

10.Employment At Will. Unless stated otherwise in a separately executed Employment Agreement between Employee and Sarepta, Employee agrees that his or her employment is "at will" which means that it can be terminated at any time by Employee or Sarepta, with or without cause and with or without notice. Employee agrees that any promise or obligation that his or her employment be on any other basis than "at will" is invalid unless in writing signed by the President of Sarepta.

11.General.

11.1 Waiver. The failure of Sarepta to claim a breach of any term of this Agreement shall not constitute a waiver of such breach or the right of Sarepta to enforce any subsequent breach of such term.

11.2 Assignment. This Agreement shall be binding on and inure to the benefit of each Party and their respective successors and assigns.

11.3 Severability. In the event that any provision of this Agreement is found to be invalid, void or unenforceable, the Parties agree that unless such provision materially affects the intent and purpose of this Agreement, such invalidity, voidability or unenforceability shall not affect the validity of this Agreement nor the remaining provisions herein.

11.4 Governing Law. Any issue or dispute arising out of or concerning this Agreement shall be covered by the laws of the Commonwealth of Massachusetts without regard to conflict of law principles and without regard to Employee's relocating to any other state in which Sarepta does business. The Parties agree that the exclusive jurisdiction for any legal action arising out of or relating to this Agreement shall be in the state or federal courts located in the Commonwealth of Massachusetts.

11.5 Entire Agreement. This Agreement, along with any non-conflicting provisions of any Employment Agreement that Employee may have signed, constitutes the entire agreement between the Parties on the subject matter hereof and supersedes all prior agreements, communications and understandings of any nature whatsoever, oral or written. In the event of any conflict, this Agreement controls. This Agreement may not be modified or waived orally and may be modified only in a writing signed by a duly authorized representative of both Parties. Nothing herein shall constitute an offer or guarantee of future employment for Employee by Sarepta.

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their duly authorized representatives and to be effective on the Effective Date.

Sarepta Therapeutics, Inc.

By: /s/ David T. Howton
Name: David T. Howton
Title: General Counsel

Date: May 23, 2018

Dr. Gilmore N. O'Neill

By: /s/ Dr. Gilmore N. O'Neill

Date: May 23, 2018

Exhibit A

Exhibit A is a list describing inventions, original works of authorship, developments, improvements, and trade secrets which were made by Employee prior to the Term (collectively referred to as “Prior Inventions”), which belong to Employee, which relate to Sarepta’s current or proposed business, products or research and development, and which are not assigned to Sarepta; or, if no such list is attached, Employee represents that there are no such Prior Inventions.

Exhibit B

Exhibit B is a list of all prior agreements now in effect under which Employee has agreed to keep information confidential or not to compete or solicit employees of any employer or person; or, if no such list is attached, Employee represents that there are no such prior agreements.

SAREPTA THERAPEUTICS, INC.

CHANGE IN CONTROL AND SEVERANCE AGREEMENT

This Change in Control and Severance Agreement (the "Agreement") is made and entered into by and between Gilmore O'Neill, M.D. (the "Executive") and Sarepta Therapeutics, Inc. (the "Company"), effective as of June 7, 2018 (the "Effective Date"), subject to and conditioned upon the Executive's commencement of employment with the Company on such date.

R E C I T A L S

A. It is expected that the Company from time to time will consider the possibility of an acquisition by another company or other change in control. The Board of Directors of the Company (the "Board") recognizes that such consideration as well as the possibility of an involuntary termination or reduction in responsibility in connection with •a change in control can be a distraction to Executive and can cause Executive to consider alternative employment opportunities. The Board has determined that it is in the best interests of the Company and its stockholders to assure that the Company will have the continued dedication and objectivity of Executive, notwithstanding the possibility, threat or occurrence of such an event.

B. The Board believes that it is in the best interests of the Company and its stockholders to provide Executive with an incentive to continue Executive's employment and to motivate Executive to maximize the value of the Company upon a Change in Control (as defined below).

C. The Board believes that it is imperative to provide Executive with severance benefits upon certain terminations of Executive's service to the Company following a Change in Control that enhance Executive's financial security and provide incentive and encouragement to Executive to remain with the Company notwithstanding the possibility of such an event.

D. Certain capitalized terms used in this Agreement are defined in Section 6 below.

The parties hereto agree as follows:

1. Term of Agreement. This Agreement shall become effective as of the Effective Date and terminate upon the date that all obligations of the parties hereto with respect to this Agreement have been satisfied.

2. At-Will Employment. Pursuant to the Employment Agreement dated as of May 23, 2018 (the "Employment Agreement"), the Company and Executive acknowledge that Executive's employment is and shall continue to be "at-will," as defined under applicable law. If Executive's employment terminates for any reason, Executive shall not be entitled to any payments, benefits, damages, awards or compensation other than as provided by this Agreement or in the Employment Agreement.

3. Covered Termination During a Change in Control Period. If Executive experiences a Covered Termination during a Change in Control Period, and if Executive delivers to the Company a general release of claims in a form acceptable to the Company (the "Release of Claims") that becomes effective and irrevocable sixty (60) days following such Covered Termination, then in addition to any Accrued Benefits payable in accordance with the Employment Agreement or applicable law, the Company shall provide Executive with the following:

(a) Severance. Executive shall be entitled to receive an amount equal to eighteen (18) months of Executive's base salary at the rate in effect immediately prior to Executive's termination of employment payable in a cash lump sum, less applicable withholdings, as soon as administratively practicable following the date the Release of Claims is not subject to revocation and, in any event, within sixty (60) days following the date of the Covered Termination.

(b) Bonus. Executive shall be entitled to receive an amount equal to one hundred percent (100%) of Executive's annual target bonus assuming achievement of performance goals at one hundred percent (100%) payable in a cash lump sum, less applicable withholdings, as soon as administratively practicable following the date the Release of Claims is not subject to revocation and, in any event, within sixty (60) days following the date of the Covered Termination.

(c) Equity Awards. Each outstanding equity award, including, without limitation, each stock option and restricted stock award, held by Executive shall automatically become vested and, if applicable, exercisable and any forfeiture restrictions or rights of repurchase thereon shall immediately lapse, in each case, with respect to one hundred percent (100%) of shares subject thereto.

(d) Continued Healthcare. If Executive elects to receive continued healthcare coverage pursuant to the provisions of COBRA, the Company shall directly pay, or reimburse Executive for, the premium for Executive and Executive's covered dependents through the earlier of (i) the eighteen (18) month anniversary of the date of Executive's termination of employment and (ii) the date Executive and Executive's covered dependents, if any, become eligible for healthcare coverage under another employer's plan(s). After the Company ceases to pay premiums pursuant to the preceding sentence, Executive may, if eligible, elect to continue healthcare coverage at Executive's expense in accordance the provisions of COBRA.

4. Other Terminations. If Executive's service with the Company is terminated by the Company or by Executive for any or no reason other than as a Covered Termination during a Change in Control Period, then Executive shall not be entitled to any benefits hereunder other than accrued but unpaid salary, bonus, vacation and expense reimbursement in accordance with applicable law and to elect any continued healthcare coverage as may be required under COBRA or similar state law.

5. Limitation on Payments. Notwithstanding anything in this Agreement to the contrary, if any payment or distribution Executive would receive pursuant to this Agreement or otherwise ("Payment") would (a) constitute a "parachute payment" within the meaning of Section 280G of the Internal Revenue Code of 1986, as amended (the "Code"), and (b) but for this sentence, be subject to the excise tax imposed by Section 4999 of the Code (the "Excise Tax"), then such Payment shall either be (i) delivered in full, or (ii) delivered as to such lesser extent which would result in no portion of such Payment being subject to the Excise Tax, whichever of the foregoing amounts, taking into account the applicable federal, state and local income taxes and the Excise Tax, results in the receipt by Executive on an after-tax basis, of the largest payment, notwithstanding that all or some portion the Payment may be taxable under Section 4999 of the Code. The accounting firm engaged by the Company for general audit purposes as of the day prior to the effective date of the Change in Control shall perform the foregoing

calculations. The Company shall bear all expenses with respect to the determinations by such accounting firm required to be made hereunder. The accounting firm shall provide its calculations to the Company and Executive within fifteen (15) calendar days after the date on which Executive's right to a Payment is triggered (if requested at that time by the Company or Executive) or such other time as requested by the Company or Executive. Any good faith determinations of the accounting firm made hereunder shall be final, binding and conclusive upon the Company and Executive. Any reduction in payments and/or benefits pursuant to this Section 5 will occur in the following order: (1) reduction of cash payments; (2) cancellation of accelerated vesting of equity awards other than stock options; (3) cancellation of accelerated vesting of stock options; and (4) reduction of other benefits payable to Executive.

6. Definition of Terms. The following terms referred to in this Agreement shall have the following meanings:

(a) Cause. "Cause" means (i) an act of dishonesty made by Executive in connection with Executive's responsibilities as an employee; (ii) Executive's conviction of, or plea of nolo contendere to, a felony or any crime involving fraud, embezzlement or any other act of moral turpitude; (iii) Executive's gross misconduct; (iv) Executive's unauthorized use or disclosure of any proprietary information or trade secrets of the Company or any other party to whom Executive owes an obligation of nondisclosure as a result of Executive's relationship with the Company; (v) Executive's willful breach of any obligations under any written agreement or covenant with the Company; or (vi) Executive's continued failure to perform his or her employment duties after Executive has received a written demand of performance from the Company which specifically sets forth the factual basis for the Company's belief that Executive has not substantially performed his or her duties and has failed to cure such non-performance to the Company's satisfaction within ten (10) business days after receiving such notice.

(b) Change in Control. “Change in Control” means the occurrence of any of the following events:

(i) Change in Ownership of the Company. A change in the ownership of the Company which occurs on the date that any one person, or more than one person acting as a group ("Person"), acquires ownership of the stock of the Company that, together with the stock held by such Person, constitutes more than fifty percent (50%) of the total voting power of the stock of the Company; provided, however, that for purposes of this subsection (i), the acquisition of additional stock by any one Person, who is considered to own more than fifty percent (50%) of the total voting power of the stock of the Company will not be considered a Change in Control; or

(ii) Change in Effective Control of the Company. If the Company has a class of securities registered pursuant to Section 12 of the Securities Exchange Act of 1934, as amended, a change in the effective control of the Company which occurs on the date that a majority of members of the Board is replaced during any twelve (12) month period by members of the Board whose appointment or election is not endorsed by a majority of the members of the Board prior to the date of the appointment or election. For purposes of this subsection (ii), if any Person is considered to be in effective control of the Company, the acquisition of additional control of the Company by the same Person will not be considered a Change in Control; or

(iii) Change in Ownership of a Substantial Portion of the Company's Assets. A change in the ownership of a substantial portion of the Company's assets which occurs on the date that any Person acquires (or has acquired during the twelve (12) month period ending on the date of the most recent acquisition by such person or persons) assets from the Company that have a total gross fair market value equal to or more than fifty percent (50%) of the total gross fair market value of all of the assets of the

Company immediately prior to such acquisition or acquisitions; provided, however, that for purposes of this subsection (iii), the following will not constitute a change in the ownership of a substantial portion of the Company's assets: (A) a transfer to an entity that is controlled by the Company's shareholders immediately after the transfer, or (B) a transfer of assets by the Company to: (1) a shareholder of the Company (immediately before the asset transfer) in exchange for or with respect to the Company's stock, (2) an entity, fifty percent (50%) or more of the total value or voting power of which is owned, directly or indirectly, by the Company, (3) a Person, that owns, directly or indirectly, fifty percent (50%) or more of the total value or voting power of all the outstanding stock of the Company, or (4) an entity, at least fifty percent (50%) of the total value or voting power of which is owned, directly or indirectly, by a Person described in this subsection (iii)(B)(3). For purposes of this subsection (iii), gross fair market value means the value of the assets of the Company, or the value of the assets being disposed of, determined without regard to any liabilities associated with such assets.

For purposes of this definition, persons will be considered to be acting as a group if they are owners of a corporation that enters into a merger, consolidation, purchase or acquisition of stock, or similar business transaction with the Company.

Further and for the avoidance of doubt, a transaction shall not constitute a Change in Control if: (i) its sole purpose is to change the state of the Company's incorporation, or (ii) its sole purpose is to create a holding company that shall be owned in substantially the same proportions by the persons who held the Company's securities immediately before such transaction.

(c) Change in Control Period. "Change in Control Period" means the six (6) month period of time immediately prior to a Change in Control through the twelve (12) month period of time commencing upon a Change in Control.

(d) Constructive Termination. "Constructive Termination" means Executive's resignation from employment with the Company within ninety (90) days after the occurrence of one or more of the following conditions without Participant's consent: (i) a material diminution in Executive's authority, duties, or responsibilities; (ii) a material diminution in Executive's base salary, other than a diminution ratably applied to other senior executives of the Company; (iii) a material change in the geographic location at which Executive must perform Executive's services hereunder (which shall in no event include a relocation of Executive's office which results in an increased commuting distance from Executive's home to the office of less than thirty (30) miles); or (iv) any other action or inaction that constitutes a material breach of this or any written agreement or covenant between Executive and the Company by the Company; and which, in the case of any of the foregoing, continues uncured by the Company beyond thirty (30) days after Executive has provided the Company written notice that Executive believes in good faith that such condition giving rise to such claim of Constructive Termination has occurred. Any such notice shall be provided to the Company within thirty (30) days following the initial occurrence of the condition or event giving rise to Constructive Termination.

(e) Covered Termination. "Covered Termination" shall mean Executive's Constructive Termination or the termination of Executive's employment by the Company other than for Cause.

7. Successors.

(a) Company's Successors. Any successor to the Company (whether direct or indirect and whether by purchase, merger, consolidation, liquidation or otherwise) to all or substantially all of the Company's business and/or assets shall assume the obligations under this Agreement and agree expressly to perform the obligations under this Agreement in the same manner and to the same extent as the Company would be required to perform such obligations in the absence of a succession. For all purposes under this Agreement, the term "Company" shall include any successor to the Company's business and/or assets which executes and delivers the assumption agreement described in this Section 7(a) or which becomes bound by the terms of this Agreement by operation of law.

(b) Executive's Successors. The terms of this Agreement and all rights of Executive hereunder shall inure to the benefit of, and be enforceable by, Executive's personal or legal representatives, executors, administrators, successors, heirs, distributees, devisees and legatees.

8. Notices. Notices and all other communications contemplated by this Agreement shall be in writing and shall be deemed to have been duly given when personally delivered or one (1) day following mailing via Federal Express or similar overnight courier service. In the case of Executive, mailed notices shall be addressed to Executive at Executive's home address that the Company has on file for Executive, with a copy to: Hollis Gonerka Bart LLP, 3 Columbus Circle, 15th Floor, New York, NY 10019. In the case of the Company, mailed notices shall be addressed to its corporate headquarters, and all notices shall be directed to the attention of its Chief Financial Officer and the Vice President of Human Resources.

9. Confidentiality; Non-Solicitation.

(a) Confidentiality. While Executive is employed by the Company, and thereafter, Executive shall not directly or indirectly disclose or make available to any person, firm, corporation, association or other entity for any reason or purpose whatsoever, any Confidential Information (as defined below). Upon termination of Executive's employment with the Company, all Confidential Information in Executive's possession that is in written or other tangible form (together with •any copies or duplicates thereof, including computer files) shall be returned to the Company and shall not be retained by Executive or furnished to any third party, in any form except as provided herein; *provided*, *however*, that Executive shall not be obligated to treat as confidential, or return to the Company copies of any Confidential Information that (i) was publicly known at the time of disclosure to Executive, (ii) becomes publicly known or available thereafter other than by any means in violation of this Agreement or any other duty owed to the Company by any person or entity, or (iii) is lawfully disclosed to Executive by a third party. For purposes of this Agreement, the term "Confidential Information" shall mean information disclosed to Executive or known by Executive as a consequence of or through his or her relationship with the Company, about the customers, employees, business methods, public relations methods, organization, procedures or finances, including, without limitation, information of or relating to customer lists, of the Company and its affiliates. In addition, Executive shall continue to be subject to any and all confidentiality and intellectual property agreements between Executive and the Company, including, without limitation, the Confidential Proprietary Rights and Non-Disclosure Agreement, (collectively, the "Confidential Information Agreements").

(b) Non-Solicitation. In addition to each Executive's obligations under the Confidential Information Agreements, Executive shall not for a period of one (1) year following Executive's termination of employment for any reason, either on Executive's own account or jointly with or as a manager, agent, officer, employee, consultant, partner, joint venturer, owner or stockholder or

otherwise on behalf of any other person, firm or corporation, directly or indirectly solicit or attempt to solicit away from the Company any of its officers or employees or offer employment to any person who is an officer or employee of the Company; *provided, however,* that a general advertisement to which an employee of the Company responds shall in no event be deemed to result in a breach of this Section 9(b). Executive also agrees not to harass or disparage the Company or its employees, clients, directors or agents or divert or attempt to divert any actual or potential business of the Company.

(c) Survival of Provisions. The provisions of this Section 9 shall survive the termination or expiration of the applicable Executive's employment with the Company and shall be fully enforceable thereafter. If it is determined by a court of competent jurisdiction in any state that any restriction in this Section 9 is excessive in duration or scope or is unreasonable or unenforceable under the laws of that state, it is the intention of the parties that such restriction may be modified or amended by the court to render it enforceable to the maximum extent permitted by the law of that state.

10. Dispute Resolution. To ensure the timely and economical resolution of disputes that arise in connection with this Agreement, Executive and the Company agree that any and all disputes, claims, or causes of action arising from or relating to the enforcement, breach, performance or interpretation of this Agreement, Executive's employment, or the termination of Executive's employment, shall be resolved to the fullest extent permitted by law by final, binding and confidential arbitration, by a single arbitrator, in Seattle, Washington, conducted by Judicial Arbitration and Mediation Services, Inc. ("JAMS") under the applicable JAMS employment rules. **By agreeing to this arbitration procedure, both Executive and the Company waive the right to resolve any such dispute through a trial by jury or judge or administrative proceeding.** The arbitrator shall: (i) have the authority to compel adequate discovery for the resolution of the dispute and to award such relief as would otherwise be permitted by law; and (ii) issue a written arbitration decision, to include the arbitrator's essential findings and conclusions and a statement of the award. The arbitrator shall be authorized to award any or all remedies that Executive or the Company would be entitled to seek in a court of law. The Company shall pay all JAMS' arbitration fees in excess of the amount of court fees that would be required if the dispute were decided in a court of law. Nothing in this Agreement is intended to prevent either Executive or the Company from obtaining injunctive relief in court to prevent irreparable harm pending the conclusion of any such arbitration. Notwithstanding the foregoing, Executive and the Company each have the right to resolve any issue or dispute over intellectual property rights by Court action instead of arbitration.

11. Miscellaneous Provisions.

(a) Section 409A.

(i) Separation from Service. Notwithstanding any provision to the contrary in this Agreement, no amount deemed deferred compensation subject to Section 409A of the Code shall be payable pursuant to Section 3 unless Executive's termination of employment constitutes a "separation from service" with the Company within the meaning of Section 409A of the Code and the Department of Treasury regulations and other guidance promulgated thereunder ("Separation from Service") and, except as provided under Section 1l(a)(ii) of this Agreement, any such amount shall not be paid, or in the case of installments, commence payment, until the sixtieth (60th) day following Executive's Separation from Service. Any installment payments that would have been made to Executive during the sixty (60) day period immediately following Executive's Separation from Service but for the preceding sentence shall be paid to Executive on the sixtieth (60th) day following Executive's Separation from Service and the remaining payments shall be made as provided in this Agreement.

(ii) Specified Employee. Notwithstanding any provision to the contrary in this Agreement, if Executive is deemed at the time of his separation from service to be a "specified employee" for purposes of Section 409A(a)(2)(B)(i) of the Code, to the extent delayed commencement of any portion of the benefits to which Executive is entitled under this Agreement is required in order to avoid a prohibited distribution under Section 409A(a)(2)(B)(i) of the Code, such portion of Executive's benefits shall not be provided to Executive prior to the earlier of (a) the expiration of the six (6)-month period measured from the date of the Executive's Separation from Service or (b) the date of Executive's death. Upon the first business day following the expiration of the applicable Code Section 409A(a)(2)(B)(i) period, all payments deferred pursuant to this Section 1l (a)(ii) shall be paid in a lump sum to Executive, and any remaining payments due under this Agreement shall be paid as otherwise provided herein.

(iii) Expense Reimbursements. To the extent that any reimbursements payable pursuant to this Agreement are subject to the provisions of Section 409A of the Code, any such reimbursements payable to Executive pursuant to this Agreement shall be paid to Executive no later than December 31 of the year following the year in which the expense was incurred, the amount of expenses reimbursed in one year shall not affect the amount eligible for reimbursement in any subsequent year, and Executive's right to reimbursement under this Agreement will not be subject to liquidation or exchange for another benefit.

(b) Waiver. No provision of this Agreement shall be modified, waived or discharged unless the modification, waiver or discharge is agreed to in writing and signed by Executive and by an authorized officer of the Company (other than Executive). No waiver by either party of any breach of, or of compliance with, any condition or provision of this Agreement by the other party shall be considered a waiver of any other condition or provision or of the same condition or provision at another time.

(c) Whole Agreement. This Agreement, the Employment Agreement and the Confidential Proprietary Rights Agreements represent the entire understanding of the parties hereto with respect to the subject matter hereof and supersede all prior arrangements and understandings regarding same.

(d) Choice of Law. The validity, interpretation, construction and performance of this Agreement shall be governed by the laws of the Commonwealth of Massachusetts.

(e) Severability. The invalidity or unenforceability of any provision or provisions of this Agreement shall not affect the validity or enforceability of any other provision hereof, which shall remain in full force and effect.

(f) Counterparts. This Agreement may be executed in counterparts, each of which shall be deemed an original, but all of which together will constitute one and the same instrument.

(Signature page follows)

IN WITNESS WHEREOF, each of the parties has executed this Agreement, in the case of the Company by its duly authorized officer, as of the day and year set forth below.

SAREPTA THERAPEUTICS, INC.

/s/ Douglas S. Ingram
By: Douglas S. Ingram

Title: President and Chief Executive Officer

Date: May 23, 2018

EXECUTIVE

/s/ Gilmore O'Neill, M.D.
By: Gilmore O'Neill, M.D.

Title: Senior Vice President, Chief Medical Officer

Date: May 23, 2018

EXHIBIT 10.4

June 26, 2018

Mr. Douglas S. Ingram

Re: Amendments to Employment Agreement and CIC Severance Agreement

Dear Doug:

This letter agreement ("Letter Agreement") amends, effective on the date above, certain terms of the Change in Control and Severance Agreement (the "CIC Severance Agreement") dated June 26, 2017 between you and Sarepta Therapeutics, Inc. (the "Company") and the Employment Agreement (the "Employment Agreement") dated June 26, 2017 between you and the Company. Capitalized terms not otherwise defined in this Letter Agreement have the meanings set forth in the CIC Severance Agreement. Except as provided herein, the CIC Severance Agreement and Employment Agreement will continue in accordance with their terms.

1. Change in Control Vesting. Notwithstanding anything to the contrary, the "Pro Rata Equity Vesting Percentage" for purposes of determining the portion of your outstanding Performance Option Award that will vest on a Covered Termination during a Change in Control Period pursuant to Section 3(c)(ii) of the CIC Severance Agreement will equal 100%. Accordingly, the portion of your Performance Option Award that will vest on such Covered Termination will not be prorated for the period in which you performed services for the Company prior to the Covered Termination.

2. Non-Competition. The noncompetition covenant set forth in Section 10(b) of the Employment Agreement will change as follows:

(a) The noncompetition covenant will extend until the later of (x) eighteen (18) months following the termination of your employment with the Company and (y) June 26, 2023.

(b) In the second sentence of Section 10(b) of the Employment Agreement, the language "and Limb-girdle muscular dystrophies" will be added after "Duchenne Muscular Dystrophy".

(c) In the second sentence of Section 10(b) of the Employment Agreement, an activity will be considered "engaged" in only if it is "actively and significantly engaged" in and an activity will be considered "planned" only if it has been "specifically planned".

(d) For the avoidance of doubt, the foregoing changes will not apply to Section 10(c) of the Employment Agreement.

3. Miscellaneous.

(a) Entire Understanding. This Letter Agreement sets forth the entire agreement between you and the Company regarding the amendment of the CIC Severance Agreement and the Employment Agreement and supersedes any other discussions or agreements between you and the Company regarding the matters addressed herein.

(b) Dispute Resolution; Governing Law. Any dispute or controversy arising under or in connection with this Letter Agreement will be settled in accordance with Section 10 (*Dispute Resolution*) of the CIC Severance Agreement. The validity, interpretation, construction and performance of this Letter Agreement will be governed by the laws of the Commonwealth of Massachusetts without regard to the choice of law principles thereof.

(c) Severability; Counterparts. The invalidity or unenforceability of any provision of this Letter Agreement will not affect the validity or enforceability of any other provision. If any provision of this Letter Agreement is held invalid or unenforceable in part, the remaining portion of such provision, together with all other provisions of this Letter Agreement, will remain valid and enforceable and continue in full force and effect to the fullest extent consistent with law. This Letter Agreement may be executed in several counterparts (including, without limitation, by facsimile, PDF or electronic transmission), each of which will be deemed an original, and such counterparts will constitute one and the same instrument.

(d) This Letter Agreement also constitutes an amendment of your Performance Stock Option Award Agreement under the Company's 2014 Employment Commencement Incentive Plan.

[*Remainder of Page Intentionally Left Blank*]

To indicate your agreement with the foregoing, please sign and return this Letter Agreement to me. This Letter Agreement will become effective as of the date on which you sign below.

Very truly yours,

SAREPTA THERAPEUTICS, INC.

By: /s/ David Tyronne Howton, Jr.
Name: David Tyronne Howton, Jr.
Title: Senior Vice President, General Counsel and Corporate Secretary

Accepted and Agreed:

/s/ Douglas S. Ingram
Name: Douglas S. Ingram
Date: June 26, 2018

617.274.4000 | 215 First Street, Cambridge, MA 02142
SAREPTA.COM

SAREPTA THERAPEUTICS, INC.

2014 EMPLOYMENT COMMENCEMENT INCENTIVE PLAN

RESTRICTED STOCK UNIT AWARD AGREEMENT

Unless otherwise defined herein, the terms defined in the 2014 Employment Commencement Incentive Plan (the "Plan") will have the same defined meanings in this Restricted Stock Unit Award Agreement (the "Award Agreement").

I. NOTICE OF RESTRICTED STOCK UNIT GRANT

Participant: [Name of Participant]

Address:

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

Date of Grant _____

Vesting Commencement Date _____

Number of RSUs _____

Vesting Schedule

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

[INSERT VESTING SCHEDULE]

Notwithstanding the foregoing, in the event of Participant's termination as an Employee as a result of the Participant's death, 100% of the RSUs will vest as of the date of such death.

Agreements and Acknowledgements

By Participant's signature and the signature of the Company's representative below, Participant and the Company agree that this award of RSUs is granted under and governed by the terms and conditions of the Plan and this Award Agreement, including the Terms and Conditions of Restricted Stock Unit Grant, attached hereto as Exhibit A, all of which are made a part of this document. Participant has reviewed the Plan and this Award Agreement in their entirety, has had an opportunity to obtain the advice of counsel prior to executing this Award Agreement and fully understands all provisions of the Plan and this Award Agreement. Participant hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Administrator upon any questions relating to the Plan and this Award Agreement. Participant agrees that Participant has not been previously employed in any capacity by the Company or a Subsidiary, or if previously employed, has had a bona-fide period of non-employment, and that the grant of this award of RSUs is an inducement material to Participant's agreement to enter into employment with the Company or Subsidiary. Participant further agrees to notify the Company upon any change in the residence address indicated below.

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

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

EXHIBIT A

TERMS AND CONDITIONS OF RESTRICTED STOCK UNIT GRANT

1. Grant of Restricted Stock Units. The Company hereby grants to the Participant named in the Notice of Grant attached as Part I of this Award Agreement (the "Participant") the number of RSUs as set forth in the Notice of Grant giving the Participant the conditional right to receive, without payment therefor, one share of Common Stock with respect to each RSU forming part of the award, subject to all of the terms and conditions in this Award Agreement and the Plan, which is incorporated herein by reference. Subject to Section 19 of the Plan, in the event of a conflict between the terms and conditions of the Plan and the terms and conditions of this Award Agreement, the terms and conditions of the Plan will prevail.

2. Vesting Schedule. Except as provided in Sections 3 ,5 and 6, the RSUs awarded by this Award Agreement will vest in accordance with the vesting provisions set forth in the Notice of Grant. RSUs scheduled to vest on a certain date or upon the occurrence of a certain condition will not vest in Participant in accordance with any of the provisions of this Award Agreement, unless Participant will have been continuously an Employee from the Date of Grant until the date such vesting occurs.

3. Administrator Discretion. The Administrator, in its discretion, may accelerate the vesting of the balance, or some lesser portion of the balance, of the unvested RSUs at any time, subject to the terms of the Plan. If so accelerated, such RSUs will be considered as having vested as of the date specified by the Administrator.

4. Company's Obligation to Pay. Subject to Section 5 below, the Company shall, as soon as practicable upon the vesting of any portion of the RSUs awarded hereunder (but in no event later than 30 days following the date on which such RSUs vest), effect delivery of the Shares with respect to such vested RSUs to the Participant (or, in the event of the Participant's death, to the Beneficiary (as defined below).

5. Forfeiture upon Termination of Relationship with Company as an Employee; Death of Participant.

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

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

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

7. Withholding of Taxes. The vesting of the RSUs awarded hereunder will give rise to "wages" subject to withholding. The Participant expressly acknowledges and agrees that the Participant's rights hereunder, including the right to be issued Shares upon vesting of the RSUs, are subject to the Participant promptly paying to the Company in cash or by check (or by such other means as may be acceptable to the Administrator) all taxes required to be withheld. No Shares will be transferred pursuant to the vesting of the RSUs unless and until the Participant has remitted to the Company an amount in cash or by check sufficient to satisfy any federal, state, or local withholding tax requirements, or has made other arrangements satisfactory to the Administrator with respect to such taxes. The Participant authorizes the Company and its subsidiaries to withhold such amount from any amounts otherwise owed to the Participant, but nothing in this sentence may be construed as relieving the Participant of any liability for satisfying his or her obligation under the preceding provisions of this Section 7.

8. Rights as Shareholder. Neither Participant nor any person claiming under or through Participant will have any of the rights or privileges of a shareholder of the Company in respect of any Shares deliverable hereunder unless and until certificates representing such Shares will have been issued, recorded on the records of the Company or its transfer agents or registrars, and delivered to Participant. After such issuance, recordation and delivery, Participant will have all the rights of a shareholder of the Company with respect to voting such Shares and receipt of dividends and distributions on such Shares.

9. No Guarantee of Employment. PARTICIPANT ACKNOWLEDGES AND AGREES THAT THE VESTING OF ANY PORTION OF THE RSUS AWARDED HEREUNDER IS EARNED ONLY BY CONTINUING AS AN EMPLOYEE AT THE WILL OF THE COMPANY (OR THE PARENT OR SUBSIDIARY EMPLOYING OR RETAINING PARTICIPANT) AND NOT THROUGH THE ACT OF BEING HIRED, BEING GRANTED THE RSUS OR BEING ISSUED SHARES HEREUNDER. PARTICIPANT FURTHER ACKNOWLEDGES AND AGREES THAT THIS AWARD AGREEMENT, THE TRANSACTIONS CONTEMPLATED HEREUNDER AND THE VESTING SCHEDULE SET FORTH HEREIN DO NOT CONSTITUTE AN EXPRESS OR IMPLIED PROMISE OF CONTINUED EMPLOYMENT FOR THE VESTING PERIOD, FOR ANY PERIOD, OR AT ALL, AND WILL NOT INTERFERE IN ANY WAY WITH PARTICIPANT'S RIGHT OR THE RIGHT OF THE COMPANY (OR THE PARENT OR SUBSIDIARY EMPLOYING OR RETAINING PARTICIPANT) TO TERMINATE PARTICIPANT'S RELATIONSHIP AS AN EMPLOYEE AT ANY TIME, WITH OR WITHOUT CAUSE.

10. Address for Notices. Any notice to be given to the Company under the terms of this Award Agreement will be addressed to the Company at Sarepta Therapeutics, Inc., 215 First Street, Suite 415, Cambridge, MA 02142, or at such other address as the Company may hereafter designate in writing.

11. Non-Transferability of RSUs. Except as provided in Section 6, this award of RSUs may not be transferred in any manner otherwise than by will or by the laws of descent or distribution.

12. Binding Agreement. Subject to the limitation on the transferability of this grant contained herein, this Award Agreement will be binding upon and inure to the benefit of the heirs, legatees, legal representatives, successors and assigns of the parties hereto.

13. Additional Conditions to Issuance of Shares. If at any time the Company will determine, in its discretion, that the listing, registration or qualification of the Shares upon any securities exchange or under any state or federal law, or the consent or approval of any governmental regulatory authority is necessary or desirable as a condition to the issuance of Shares to Participant (or his or her estate), such issuance will not occur unless and until such listing, registration, qualification, consent or approval will have been effected or obtained free of any conditions not acceptable to the Company. The Company will make all reasonable efforts to meet the requirements of any such state or federal law or securities exchange and to obtain any such consent or approval of any such governmental authority. Participant acknowledges that the Plan is intended to conform with the requirements of rules promulgated by the Nasdaq Stock Market and, without limiting the foregoing, in particular Nasdaq Stock Market Rule 5635(c).

14. Plan Governs. This Award Agreement is subject to all terms and provisions of the Plan. In the event of a conflict between one or more provisions of this Award Agreement and one or more provisions of the Plan, the provisions of the Plan will govern. Capitalized terms used and not defined in this Award Agreement will have the meaning set forth in the Plan.

15. Administrator Authority. The Administrator will have the power to interpret the Plan and this Award Agreement and to adopt such rules for the administration, interpretation and application of the Plan as are consistent therewith and to interpret or revoke any such rules (including, but not limited to, the determination of whether or not any RSUs forming part of the award have vested). All actions taken and all interpretations and determinations made by the Administrator in good faith will be final and binding upon Participant, the Company and all other interested persons. No member of the Administrator will be personally liable for any action, determination or interpretation made in good faith with respect to the Plan or this Award Agreement. In its absolute discretion, the Board may at any time and from time to time exercise any and all rights and duties of the Committee under the Plan and this Award Agreement; *provided*, *however*, any action taken by the Board in connection with the administration of the Plan shall not be deemed approved by the Board unless such actions are approved by a majority of the Outside Directors.

16. Electronic Delivery. The Company may, in its sole discretion, decide to deliver any documents related to RSUs awarded under the Plan by electronic means or request Participant's consent to participate in the Plan by electronic means. Participant hereby consents to receive such documents by electronic delivery and agrees to participate in the Plan through any on-line or electronic system established and maintained by the Company or another third party designated by the Company.

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

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

19. Modifications to the Agreement. This Award Agreement constitutes the entire understanding of the parties on the subjects covered. Participant expressly warrants that he or she is not accepting this Award Agreement in reliance on any promises, representations, or inducements other than those contained herein. Modifications to this Award Agreement or the Plan can be made only in an express written contract executed by a duly authorized officer of the Company. This Award Agreement and the award of RSUs hereunder is intended to be exempt from Section 409A of the Code. Notwithstanding anything to the contrary in the Plan or this Award Agreement, the Company reserves the right to revise this Award Agreement as it deems necessary or advisable, in its sole discretion and without the consent of Participant, to comply with Section 409A of the Code, or to otherwise avoid imposition of any additional tax or income recognition under Section 409A of the Code in connection with this award of RSUs.

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

21. Amendment, Suspension or Termination of the Plan. By accepting this Award, Participant expressly warrants that he or she has received an award of RSUs under the Plan, and has received, read and understood a description of the Plan. Participant understands that the Plan is discretionary in nature and may be amended, suspended or terminated by the Company at any time.

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

23. Shareholder Approval Not Required. The Plan will not be submitted for approval by the Company's shareholders. As more particularly described in Section 19(b) of the Plan, pursuant to Nasdaq Stock Market Rule 5635(c), the issuance of this award of RSUs and the Shares to be delivered following the vesting of such RSUs pursuant to the Plan are not subject to the approval of the Company's shareholders.

EXHIBIT 10.6

AMENDMENT NO. 2
TO THE
SAREPTA THERAPEUTICS, INC.
2014 EMPLOYMENT COMMENCEMENT INCENTIVE PLAN

WHEREAS, Sarepta Therapeutics, Inc. (the "Company") previously adopted and approved the 2014 Employment Commencement Incentive Plan (the "Plan") as an inducement stock plan under Nasdaq Stock Market Rule 5635(c)(4) to, among other things, attract and retain the best candidates for positions of substantial responsibility upon whose judgment, interest, and special effort the successful conduct of the Company's operation will be largely dependent; and

WHEREAS, pursuant to Sections 19(a) and (b) of the Plan, the "Administrator" (defined under the Plan as the Board of Directors of the Company (the "Board") or any of its committees) may amend the Plan from time to time to time without stockholder approval; and

WHEREAS, the Compensation Committee of the Board (the "Committee"), as Administrator, has determined that it is in the best interests of the Company to amend the Plan, to increase the number of authorized shares under the Plan by 1,150,000 shares of common stock of the Company, as authorized under the Plan;

NOW, THEREFORE, the Plan hereby is amended, effective July 26, 2018, the date of approval by the Committee, as follows:

1. Section 3(a) of the Plan, entitled "Stock Subject to the Plan," shall be replaced in its entirety by the following:

"Subject to the provisions of Section 14(a) of the Plan, the maximum aggregate number of Shares that may be subject to Awards and sold under the Plan is 6,590,000 Shares; provided, however, that such aggregate number of Shares available for issuance under the Plan shall be reduced by 1.41 Shares for each Share delivered in settlement of any Full Value Award. The Shares may be authorized, but unissued, or reacquired Common Stock."

2. Except as modified herein, the Plan is hereby specifically ratified and affirmed.

This Amendment No. 2 to the Plan is adopted by the Committee, effective as of the date of approval by the Committee.

IN WITNESS WHEREOF, this Amendment has been executed by its duly authorized officer on August 7, 2018.

SAREPTA THERAPEUTICS, INC.

By: /s/ David Tyronne Howton, Jr.
Name: David Tyronne Howton, Jr.
Title: Senior Vice President, Corporate Secretary and General Counsel

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 8, 2018

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

EXHIBIT 31.2

CERTIFICATION

I, Sandesh Mahatme, 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 8, 2018

/s/ SANDESH MAHATME

Sandesh Mahatme
Executive Vice President,
Chief Financial Officer and
Chief Business 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, 2018, 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 8, 2018

/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, Sandesh Mahatme, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended June 30, 2018 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 8, 2018

/s/ SANDESH MAHATME
Sandesh Mahatme
Executive Vice President,
Chief Financial Officer and
Chief Business 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.