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 March 31, 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	**65,531,046**
(Class)	**(Outstanding as of April 30, 2018)**

SAREPTA THERAPEUTICS, INC.
FORM 10-Q
INDEX

		Page
PART I — FINANCIAL INFORMATION		
Item 1.	Financial Statements (unaudited)	3
	Condensed Consolidated Balance Sheets — As of March 31, 2018 and December 31, 2017	3
	Condensed Consolidated Statements of Operations and Comprehensive Loss — For the Three Months Ended March 31, 2018 and 2017	4
	Condensed Consolidated Statements of Cash Flows — For the Three Months Ended March 31, 2018 and 2017	5
	Notes to Unaudited Condensed Consolidated Financial Statements	6
Item 2.	Management's Discussion and Analysis of Financial Condition and Results of Operations	20
Item 3.	Quantitative and Qualitative Disclosures about Market Risk	31
Item 4.	Controls and Procedures	31
PART II — OTHER INFORMATION		
Item 1.	Legal Proceedings	32
Item 1A.	Risk Factors	32
Item 2.	Unregistered Sales of Equity Securities and Use of Proceeds	58
Item 3.	Defaults Upon Senior Securities	58
Item 4.	Mine Safety Disclosures	58
Item 5.	Other Information	58
Item 6.	Exhibits	59
Exhibits		60
Signatures		61

Item 1. Financial Statements

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

	As of March 31, 2018	As of December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 557,234	$ 599,691
Short-term investments	491,757	479,369
Accounts receivable	39,848	29,468
Inventory	99,375	83,605
Other current assets	31,203	36,511
Total current assets	1,219,417	1,228,644
Property and equipment, net of accumulated depreciation of $19,817 and $18,022 as of March 31, 2018 and December 31, 2017, respectively	53,927	43,156
Intangible assets, net of accumulated amortization of $4,659 and $4,145 as of March 31, 2018 and December 31, 2017, respectively	14,473	14,355
Other assets	12,466	21,809
Total assets	$ 1,300,283	$ 1,307,964
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 17,379	$ 8,467
Accrued expenses	65,648	68,982
Current portion of long-term debt	3,446	6,175
Other current liabilities	4,723	4,708
Total current liabilities	91,196	88,332
Long-term debt	427,365	424,876
Deferred rent and other	4,962	5,539
Total liabilities	523,523	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; 65,493,293 and 64,791,670 issued and outstanding at March 31, 2018 and December 31, 2017, respectively	7	6
Additional paid-in capital	2,029,767	2,006,598
Accumulated other comprehensive loss	(643)	(379)
Accumulated deficit	(1,252,371)	(1,217,008)
Total stockholders' equity	776,760	789,217
Total liabilities and stockholders' equity	$ 1,300,283	$ 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 March 31,	
	2018	**2017**
Revenues:		
Product, net	$ 64,604	$ 16,342
Total revenues	64,604	16,342
Costs and expenses:		
Cost of sales (excluding amortization of in-licensed rights)	5,582	223
Research and development	46,204	29,119
Selling, general and administrative	43,341	26,216
Amortization of in-licensed rights	216	29
Total costs and expenses	95,343	55,587
Operating loss	(30,739)	(39,245)
Other (loss) income:		
Gain from sale of Priority Review Voucher	—	125,000
Interest (expense) income and other, net	(4,485)	335
Other (loss) income	(4,485)	125,335
(Loss) income before income tax expense	(35,224)	86,090
Income tax expense	139	2,000
Net (loss) income	(35,363)	84,090
Other comprehensive (loss) income:		
Unrealized (loss) gain on cash equivalents and short-term investments	(264)	65
Total other comprehensive (loss) income	(264)	65
Comprehensive (loss) income	$ (35,627)	$ 84,155
Net (loss) income per share		
Basic (loss) earnings per share	$ (0.55)	$ 1.53
Diluted (loss) earnings per share	$ (0.55)	$ 1.50
Weighted average number of shares of common stock used in computing:		
Basic (loss) earnings per share	64,631	54,850
Diluted (loss) earnings per share	64,631	56,012

See accompanying notes to unaudited condensed consolidated financial statements.

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

	For the Three Months Ended March 31,	
	2018	2017
Cash flows from operating activities:		
Net (loss) income	$ (35,363)	$ 84,090
Adjustments to reconcile net loss to cash flows from operating activities:		
Gain from sale of Priority Review Voucher	—	(125,000)
Depreciation and amortization	2,252	1,637
Amortization of investment discount	(1,259)	(100)
Non-cash interest expense	4,940	82
Loss on disposal of assets	10	485
Stock-based compensation	10,526	5,712
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable	(10,380)	(7,050)
Net increase in inventory	(15,770)	(17,632)
Net decrease in other assets	4,672	774
Net increase (decrease) in accounts payable, accrued expenses, deferred revenue and other liabilities	4,704	(886)
Net cash used in operating activities	(35,668)	(57,888)
Cash flows from investing activities:		
Purchase of property and equipment	(12,166)	(4,465)
Purchase of intangible assets	(673)	(1,245)
Purchase of available-for-sale securities	(91,514)	—
Proceeds from sale of Priority Review Voucher	—	125,000
Maturity of restricted investment	—	10,695
Maturity and sale of available-for-sale securities	90,093	80,000
Net cash (used in) provided by investing activities	(14,260)	209,985
Cash flows from financing activities:		
Proceeds from revolving line of credit	96,235	—
Payments on mortgage loans	(1,265)	(43)
Payment of term loan	—	(2,500)
Payments on revolving line of credit	(100,142)	—
Proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program	12,643	2,749
Net cash provided by financing activities	7,471	206
(Decrease) increase in cash and cash equivalents	(42,457)	152,303
Cash, cash equivalents and restricted cash:		
Beginning of period	599,827	122,556
End of period	557,370	274,859
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 853	$ 291
Supplemental schedule of non-cash investing activities and financing activities:		
Shares withheld for taxes	$ —	$ 165
Reclassification of long term investments to short term investments	$ 9,980	$ —
Intangible assets included in accrued expenses	$ 202	$ 179
Accrual for debt issuance costs related to the term loans	$ —	$ 400
Property and equipment included in accrued expenses	$ 2,980	$ 330

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. The Company continues to work with the EMA during their review process and anticipate they will complete their review and make a final decision on the approvability of the Company's MAA for eteplirsen in the first half of 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 has commenced shipments through the MAP and continue to expand the MAP to include more countries. 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 March 31, 2018, the Company had approximately $1,049.8 million of cash, cash equivalents and investments, consisting of $557.2 million of cash and cash equivalents, $491.8 million of short-term investments, and $0.8 million of restricted cash and investments. 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 March 31, 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%, 34% and 18% of net product revenues and 60%, 22% and 9% of accounts receivable from product sales, respectively. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profile. As of March 31, 2018, the Company believes that such customers are of high credit quality.

As of March 31, 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 the three months ended March 31, 2018, 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 March 31, 2018.

Recent Accounting Pronouncements

In February 2016, the Financial Accounting Standards Board 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 March 31, 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 Financial Accounting Standards Board 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 March 31, 2018, we are currently evaluating the potential impact that this new standard may have on our financial position and results of operations.

Reclassification

The Company has revised the presentation as well as the caption of certain items within the unaudited condensed consolidated balance sheets to conform to the current period presentation. "Restricted cash and investments" of $0.8 million and

"deferred revenue" of $3.3 million as December 31, 2017 are grouped into "other assets" and "other current liabilities", respectively. These revisions had no impact on total assets nor total liabilities.

Additionally, the Company has revised the presentation as well as caption of certain items within the unaudited condensed consolidated statements of operations and comprehensive loss to conform to the current period presentation. "Amortization of in-licensed rights" of less than $0.1 million was reclassified from "cost of sales" and presented separately in the unaudited condensed consolidated statements of operations and comprehensive loss. The reclassification had no impact on operating loss or net income.

Furthermore, the Company has also revised the presentation as well as caption of certain items within the unaudited condensed consolidated statements of cash flows to conform to the current period presentation. "Accretion of discount on available-for-sale securities" of $0.1 million and "Non-cash interest expense" of approximately $0.1 million are presented separately in the unaudited condensed consolidated statements of cash flows. These revisions had no impact on the net cash used in operating activities or cash, cash equivalents and restricted cash at end of period.

3. 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 March 31, 2018			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Cash equivalents	$ 370,052	$ 370,052	$ —	$ —
Commercial paper	178,875	—	178,875	—
Government and government agency bonds	270,119	270,119	—	—
Corporate bonds	92,195	92,195	—	—
Certificates of deposit	648	648	—	—
Total assets	$ 911,889	$ 733,014	$ 178,875	$ —

	Fair Value Measurement as of December 31, 2017			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Cash equivalents	$ 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 assets	$ 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 March 31, 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.

4. 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 March 31, 2018	As of December 31, 2017
	(in thousands)	
Money market funds	370,052	352,370
Corporate bonds	24,451	16,720
Government and government agency bonds	24,981	49,972
Total	419,484	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 March 31, 2018 and December 31, 2017 was approximately three 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 March 31, 2018			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 507,802	$ —	$ —	$ 507,802
Commercial paper	178,875	—	—	178,875
Government and government agency bonds	270,422	1	(304)	270,119
Corporate bonds	92,535	—	(340)	92,195
Total assets	$ 1,049,634	$ 1	$ (644)	$ 1,048,991
As reported:				
Cash and cash equivalents	$ 557,233	$ 1	$ —	$ 557,234
Short-term investments	$ 492,401	$ —	$ (644)	$ 491,757
Total assets	$ 1,049,634	$ 1	$ (644)	$ 1,048,991

	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 assets	$ 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 assets	$ 1,089,419	$ 2	$ (381)	$ 1,089,040

5. 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 March 31, 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 March 31, 2018	As of December 31, 2017
	(in thousands)	
Product sales, net of discounts and allowances	$ 38,919	$ 28,539
Government contract receivables	929	929
Total accounts receivable	$ 39,848	$ 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	2,790	5,159	495	1,534	9,978
Payments/credits	(2,916)	(989)	(435)	(146)	(4,486)
Balance, as of March 31, 2018	$ 869	$ 11,129	$ 229	$ 1,852	$ 14,079

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

	As of March 31, 2018	As of December 31, 2017
	(in thousands)	
Reduction to accounts receivable	$ 1,367	$ 1,285
Component of accrued expenses	12,712	7,302
Total reserves	$ 14,079	$ 8,587

6. 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 March 31, 2018	As of December 31, 2017
	(in thousands)	
Raw materials	$ 63,494	$ 53,875
Work in progress	34,609	27,442
Finished goods	1,272	2,288
Total inventory	$ 99,375	$ 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.

7. ASSET HELD FOR SALE

The Company owned a facility located at 1749 SW Airport Avenue, Corvallis, OR ("Airport Facility"). The Airport Facility was previously leased to an unrelated third party. In July 2016, the third party lessee terminated the lease and vacated the facility. The Company set up a program to actively market the Airport Facility. Accordingly, the Airport Facility with net book value of approximately $1.5 million was reclassified as an asset held for sale which is presented as a component of other current assets. In August 2017, the Company entered into a purchase and sale agreement with an unrelated third-party buyer. The sale price of as well as fees related to the Airport Facility were approximately $1.5 million and $0.2 million, respectively. The transaction was completed in January 2018 when cash was received and the two mortgage loans associated with the facility were repaid. For more information regarding the two mortgage loans, please read *Note 10, Indebtedness.*

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 March 31, 2018	As of December 31, 2017
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 15,049	$ 18,650
Prepaid clinical and preclinical expenses	6,103	5,175
Prepaid maintenance and license fees	2,442	1,711
Prepaid research expenses	2,140	2,896
Prepaid commercial expenses	883	1,589
Asset held for sale	—	1,501
Other prepaids	2,186	2,726
Other	2,400	2,263
Total other current assets	$ 31,203	$ 36,511

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

	As of March 31, 2018	As of December 31, 2017
	(in thousands)	
Prepaid clinical expenses	$ 6,151	$ 7,488
Alternative minimum tax credit	3,315	3,315
Manufacturing-related deposits	1,975	—
Restricted cash and investments	784	784
Long-term available-for-sale securities	—	9,980
Other	241	242
Total other non-current assets	$ 12,466	$ 21,809

9. ACCRUED EXPENSES

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

	As of March 31, 2018	As of December 31, 2017
	(in thousands)	
Product revenue related reserves	$ 12,712	$ 7,302
Accrued clinical and preclinical costs	10,924	15,975
Accrued professional fees	10,351	6,794
Accrued employee compensation costs	7,906	14,402
Accrued contract manufacturing costs	7,025	14,019
Accrued interest expense	3,411	1,291
Accrued collaboration cost sharing	3,197	—
Accrued BioMarin royalties	3,117	2,846
Accrued property and equipment	2,980	2,525
Accrued income taxes	943	943
Accrued research costs	226	401
Other	2,856	2,484
Total accrued expenses	$ 65,648	$ 68,982

10. INDEBTEDNESS

2024 Convertible Notes

On November 14, 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 $713.0 million as of March 31, 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 March 31, 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 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.

Mortgage Loans

The Company had two loans outstanding which bear interest at 4.75%, mature in February 2027 and are collateralized by the Airport Facility in Corvallis, Oregon. In connection with the sale of the Airport Facility in January 2018, the two long-term mortgage loans were repaid.

As of March 31, 2018, the Company recorded approximately $3.4 million as current portion of long-term debt and approximately $427.4 million as long-term debt on the unaudited condensed consolidated balance sheets related to the 2024 convertible notes, the term loan, the revolving line of credit and the mortgage loans. The following table summarizes the Company's debt facilities for the periods indicated:

	As of March 31, 2018	As of December 31, 2017
	(in thousand)	
Par value of the 2024 Notes	570,000	570,000
Unamortized discounts	(154,340)	(158,890)
Debt issuance expenses	(10,151)	(10,450)
Net carrying value of convertible debt	405,509	400,660
Other debt facilities	25,302	30,391
Net carrying value of total debt facilities	430,811	431,051

For the three months ended March 31, 2018 and 2017, the Company recorded $7.6 million and $0.3 million in interest expense, respectively.

11. RESTRUCTURING

In March 2016, the Company announced a long-term plan ("Corvallis 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. As of March 31, 2018, the Company continues to be obligated to make $4.5 million of minimum lease payments and certain other contractual maintenance costs for the whole facility.

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

	For the Three Months Ended March 31, 2018	For the Year Ended 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	(355)	(1,675)
Restructuring reserve ending balance	$ 2,578	$ 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 March 31,			
	2018		2017	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options	1,124,585	$ 34.08	877,492	$ 16.30
Restricted stock units	150,725	$ 71.45	161,029	$ 32.63
Restricted stock awards	17,090	$ 71.45	6,500	$ 31.06

Stock-based Compensation Expense

For the three months ended March 31, 2018 and 2017, total stock-based compensation expense was $10.5 million and $5.7 million, respectively. The following table summarizes stock-based compensation expense by function included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended March 31,	
	2018	2017
	(in thousands)	
Research and development	$ 2,060	$ 1,874
Selling, general and administrative	8,466	3,838
Total stock-based compensation expense	$ 10,526	$ 5,712

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 March 31,	
	2018	2017
	(in thousands)	
Stock options	$ 8,973	$ 5,038
Restricted stock awards/units	1,260	204
Employee stock purchase plan ("ESPP")	293	470
Total stock-based compensation expense	$ 10,526	$ 5,712

13. OTHER INCOME AND LOSS

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

	For the Three Months Ended March 31,	
	2018	2017
	(in thousand)	
Interest expenses	$ (7,634)	$ (257)
Interest income	1,872	186
Amortization of investment discount	1,259	100
Other income	18	306
Gain from sale of Priority Review Voucher	—	125,000
Total other (loss) income	$ (4,485)	$ 125,335

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.

For the three months ended March 31, 2018, the Company recorded a provision for income taxes of $0.1 million, representing an effective tax rate of 0.4%. The Company's estimated annual effective tax rate is lower than the federal statutory rate due to the jurisdictional mix of earnings and the release of valuation allowance against its federal and state tax attributes which can be used to offset current year earnings.

For the three months ended March 31, 2017, the Company recorded a provision for income taxes of $2.0 million, representing an effective tax rate of 2.3%. The Company computed its tax provision for the three months ended March 31, 2017 based upon the year-to-date effective tax rate as opposed to an estimated annual effective tax rate. The Company concluded that the year-to-date effective tax rate was the most appropriate method to use for the three months ended March 31, 2017, given a reliable estimate of the annual effective tax rate could not be made.

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. Given that the Company generated a net loss for the three month period ended March 31, 2018, there is no difference between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive and, therefore, would be excluded from the diluted net loss per share calculation.

	For the Three Months Ended March 31,	
	2018	2017
	(in thousands, except per share amounts)	
Net (loss) income	$ (35,363)	$ 84,090
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	64,631	54,850
Dilutive effect of outstanding stock awards and stock options after application of the treasury stock method*	—	1,162
Weighted-average number of shares of common stock and dilutive common stock equivalents outstanding for computing diluted loss per share	64,631	56,012
Net (loss) earnings per share - basic and diluted		
Basic (loss) earnings per share	$ (0.55)	$ 1.53
Diluted (loss) earnings per share	$ (0.55)	$ 1.50

* For the three months ended March 31, 2018, stock options, RSAs, RSUs, stock appreciation rights and potentially issuable stock for ESPP to purchase 9.9 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 March 31, 2017, out of money stock options, unvested performance-based RSUs and RSAs whose performance milestones were not achieved and potentially issuable stock for ESPP to purchase approximately 3.4 million were excluded from the net earnings per share calculation as their effect would have been anti-dilutive.

16. COMMITMENTS AND CONTINGENCIES

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. Plaintiffs did not file a petition for rehearing before the operative deadline. As such, the risk of loss is negligible, barring a successful and highly unlikely petition for a writ of certiorari from the U.S. Supreme Court.

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

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

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 and do not believe that disposition of the McDonald suit will have a material financial impact on the Company. The parties have now completed the confirmatory discovery process that will allow plaintiffs' counsel to represent to the court that the terms of the settlement are fair.

17. SUBSEQUENT EVENTS

On April 27, 2018, the Company entered into a seventh amendment (the "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 Company expects to incur an incremental $54.4 million in rent expense as a result of the Amendment through the remainder of the extended lease term.

On May 3, 2018, the Company and Myonexus Therapeutics, Inc. (“Myonexus”) entered into a warrant to purchase common stock of Myonexus, which, in combination with amendments to the Myonexus certificate of incorporation, provides the Company with an exclusive option to acquire Myonexus. The Company may exercise the option at any time prior to the 60th day after Myonexus: (i) completes 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 of Myonexus’ Phase 1/2A clinical trial of MYO-101, and (ii) provides the Company with the study’s final biopsy results, subject to certain conditions. The Company is liable for a $60 million non-refundable up-front payment upon execution of the agreement and may be obligated to make up to $45 million in development milestone payments. An additional payment would be required if and when the Company exercises the exclusive option to acquire Myonexus.

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 possibility of a re-examination of our marketing authorization application ("MAA") for eteplirsen being granted by the Committee for Medicinal Products for Human Use ("CHMP") and a Scientific Advisory Group ("SAG") being convened.*
- *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;*
- *the impact of regulations and regulatory decisions 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 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;*
- *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.;*
- *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;
- 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; and
- 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.

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. Preclinical 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 manufacturing and techniques 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 products and optimizing manufacturing for PPMO. We have entered into certain manufacturing and supply arrangements with third-party suppliers which will in part utilize these techniques to support production of certain of our product candidates and their components. 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 manufacturing capabilities to produce our product and product candidates for commercial and/or clinical use.

Cash, Cash Equivalents and Investments

As of March 31, 2018, we had approximately $1,049.8 million of cash, cash equivalents and investments, consisting of $557.2 million of cash and cash equivalents, $491.8 of short term investments, and $0.8 million of long-term restricted cash and investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve months.

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

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 Months Ended March 31, 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 March 31,			
	2018	2017	Change	Change
	(in thousands, except share and per share amounts)		$	%
Revenues:				
Product, net	$ 64,604	$ 16,342	$ 48,262	295%
Total revenues	64,604	16,342	48,262	295%
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	5,582	224	5,358	NM*
Research and development	46,204	29,119	17,085	59%
Selling, general and administrative	43,341	26,216	17,125	65%
Amortization of in-licensed rights	216	28	188	NM*
Total cost and expenses	95,343	55,587	39,756	72%
Operating loss	(30,739)	(39,245)	8,506	(22)%
Other (loss) income:				
Gain from Sale of Priority Review Voucher	—	125,000	(125,000)	(100)%
Interest (expense) income and other, net	(4,485)	335	(4,820)	NM*
Other (loss) income before income tax expense	(35,224)	86,090	(121,314)	(141)%
Income tax expense	139	2,000	(1,861)	(93)%
Net (loss) income	$ (35,363)	$ 84,090	$ (119,453)	(142)%
Net (loss) income per share:				
Basic (loss) earnings per share	$ (0.55)	$ 1.53	$ (2.08)	(136)%
Diluted (loss) earnings per share	$ (0.55)	$ 1.50	$ (2.05)	(136)%
Weighted average number of shares of common stock used in calculating:				
Basic (loss) earnings per share	64,631	54,850		
Diluted (loss) earnings per share	64,631	56,012		

* 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 months ended March 31, 2018 increased by $48.3 million compared with the three months ended March 31, 2017. The increase primarily reflects 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 payment to BioMarin Pharmaceutical, Inc. (“BioMarin”) as a result of the execution of the settlement and licenses agreements in July 2017 and inventory costs that relate to sales of EXONDYS 51 following our commercial launch in the U.S. Prior to receiving regulatory approval for EXONDYS 51 from the FDA in September 2016, we expensed such manufacturing and material costs as research and development expenses. For EXONDYS 51 sold in the three months ended March 31, 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 $6.8 million and $1.0 million for the three months ended March 31, 2018 and 2017, respectively.

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

	For the Three Months Ended March 31,			
	2018	2017	Change	Change
	(in thousands)		$	%
Royalty payments to BioMarin	$ 3,117	$ —	3,117	NA
Inventory costs related to EXONDYS 51 sold	1,520	8	1,512	NA
Overhead costs	943	197	746	379%
Other inventory costs	2	18	(16)	NM*
Total cost of sales	$ 5,582	$ 223	5,359	NM*

* NM = Not Meaningful

The cost of sales for the three months ended March 31, 2018, increased $5.4 million compared with the same period in 2017. The increase 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 preclinical 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. We are currently conducting various clinical trials for EXONDYS 51. We completed Part I and are conducting Part II of a Phase 1/2a clinical trial for an exon 53-skipping product candidate in the EU. We have completed the dose titration portion and are conducting the open-label portion of a study for our exon 45-skipping product candidate. A placebo-controlled study with product candidates designed to skip exons 45 and 53 is ongoing in the US, the EU, Canada, and Israel and we plan to have sites in additional countries globally. The remainder of our research and development programs are in various stages of research and preclinical 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 March 31,			
	2018	2017	Change	Change
	(in thousands)		$	%
Golodirsen (exon 53)	$ 8,505	$ 3,082	$ 5,423	176%
Casimersen (exon 45)	6,196	3,422	2,774	81%
Eteplirsen (exon 51)	4,928	8,930	(4,002)	(45)%
PPMO platform	3,724	1,253	2,471	197%
Utrophin (Summit collaboration cost sharing)	3,197	—	3,197	NA
Other projects	135	194	(59)	(30)%
Internal research and development expenses	19,519	12,238	7,281	59%
Total research and development expenses	$ 46,204	$ 29,119	$ 17,085	59%

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 $1.3 million for the three months ended March 31, 2017 was reclassified from “other projects” and presented separately in the table to conform to current year presentation.

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

	For the Three Months Ended March 31,			
	2018	2017	Change	Change
	(in thousands)		$	%
Clinical and manufacturing expenses	$ 18,764	$ 14,362	$ 4,402	31%
Compensation and other personnel expenses	8,349	5,686	2,663	47%
Professional services	4,572	2,162	2,410	111%
Utrophin (Summit collaboration cost sharing)	3,197	—	3,197	NA
Preclinical expenses	3,082	1,524	1,558	102%
Facility-related expenses	2,861	2,208	653	30%
Stock-based compensation	2,060	1,874	186	10%
Research and other	3,319	1,303	2,016	155%
Total research and development expenses	$ 46,204	$ 29,119	$ 17,085	59%

Research and development expenses for the three months ended March 31, 2018 increased by $17.1 million, or 59%, compared with the three months ended March 31, 2017. The increase was primarily driven by the following:

- $4.4 million increase in clinical and manufacturing expenses primarily due to increased patient enrollment in our ongoing clinical trials in golodirsen and casimersen as well as a ramp-up of manufacturing activities for 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;
- $3.2 million increase in collaboration cost sharing with Summit on its Utrophin platform
- $2.7 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $2.4 million increase in professional services primarily due to accelerated company growth as a result of expansion of our R&D pipeline; and
- $1.6 million increase in preclinical expenses primarily due to the continuing ramp-up of toxicology studies in our PPMO platform as well as golodirsen and casimersen.

Selling, General and Administrative Expenses

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

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

	For the Three Months Ended March 31,			
	2018	2017	Change	Change
	(in thousands)		$	%
Professional services	$ 16,154	9,741	$ 6,413	66%
Compensation and other personnel expenses	14,095	8,779	5,316	61%
Stock-based compensation	8,466	3,838	4,628	121%
Facility-related expenses	2,041	1,806	235	13%
Other	2,585	2,052	533	26%
Total selling, general and administrative expenses	$ 43,341	$ 26,216	$ 17,125	65%

The Company has revised the presentation as well as certain captions in the selling, general and administrative expenses table presented above. For the three months ended March 31, 2017, “facility-related expenses” of $1.8 million were reclassified from “other” and presented separately in the table to conform to current year presentation.

Selling, general and administrative expenses for the three months ended March 31, 2018 increased by $17.1 million, or 65%, compared with the three months ended March 31, 2017. This was primarily driven by the following:

- $6.4 million increase in professional services primarily due to continuing global expansion as well as preparation for a potential product launch in the EU should our MAA be approved by the EMA;
- $5.3 million increase in compensation and other personnel expenses primarily due to a net increase in headcount; and
- $4.6 million increase in stock-based compensation primarily due to an increase in stock price and the impact of revising the forfeiture rate assumption for officers and members of our Board of Directors (“directors”). Historically, we applied one forfeiture rate assumption to all employees and directors. Beginning on January 1, 2018, based on recent trending of employee turnover data, we began to apply different forfeiture rates to non-officer employees and directors and officers.

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 months ended March 31, 2018 and 2017, we recorded amortization of in-licensed rights of approximately $0.2 million and less than $0.1 million, respectively.

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 income (expense) and other, net

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

For the three months ended March 31, 2018, interest expense and other, net was approximately $4.5 million. For the three months ended March 31, 2017, interest income and other, net was approximately $0.3 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

Income tax expense for the three months ended March 31, 2018 was approximately $0.1 million which related to minimum state tax payments. Income tax expense for the three months ended March 31, 2017 was approximately $2.0 million which related to alternative minimum tax due to 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 March 31, 2018	As of December 31, 2017	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 557,234	$ 599,691	$ (42,457)	(7)%
Short-term investments	491,757	479,369	12,388	3%
Long-term investments	—	9,980	(9,980)	(100)%
Restricted cash and investments	784	784	—	—
Total cash, cash equivalents and investments	$ 1,049,775	$ 1,089,824	$ (40,049)	(4)%
Borrowings:				
Current portion of long-term debt	$ 3,446	$ 6,175	$ (2,729)	(44)%
Long-term debt	427,365	400,641	26,724	7%
Total borrowings	$ 430,811	$ 406,816	$ 23,995	6%
Working capital				
Current assets	$ 1,219,417	$ 1,228,644	$ (9,227)	(1)%
Current liabilities	91,196	88,332	2,864	3%
Total working capital	$ 1,128,221	$ 1,140,312	$ (12,091)	(1)%

For the period ended March 31, 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 preclinical trials;
- the attainment of milestones and our obligations to make milestone payments to 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 or the licensing or sale of our technologies. We cannot provide assurances that financing will be available when and as needed or that, if available, the financings will be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we require, this would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

Cash Flows

	For the Three Months Ended March 31,			
	2018	2017	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ (35,668)	$ (57,888)	$ 22,220	(38)%
Investing activities	(14,260)	209,985	(224,245)	(107)%
Financing activities	7,471	206	7,265	NM*
Increase in cash and cash equivalents	$ (42,457)	$ 152,303	$ (194,760)	(128)%

*NM = Not Meaningful

Operating Activities. Cash used in operating activities decreased by $22.2 million for the three months ended March 31, 2018 compared with the three months ended March 31, 2017. This was primarily driven by an increase of $8.7 million in non-cash adjustments, favorable changes of $8.0 million in operating assets and liabilities primarily due to timing of certain payments and a decrease of $5.5 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 and selling, general and administrative expenses.

Investing Activities. The cash used in investing activities for the three months ended March 31, 2018 was $14.3 million and the cash provided by investing activities for the three months ended March 31, 2017 was $210.0 million. The unfavorable change was driven by proceeds of $125.0 million from sale of the PRV and $10.7 million in maturity of restricted investment in March 2017 and increases of $91.5 million in purchase of available-for-sale securities and $7.7 million in purchase of property and equipment. These were partially offset by an increase of $10.0 million from the maturity of available-for-sale securities.

Financing Activities. Cash provided by financing activities increased by $7.3 million for the three months ended March 31, 2018 compared with the three months ended March 31, 2017. This was primarily driven by increases of $9.9 million in proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program partially offset by an increase of $2.6 million in net repayment of the debt facilities.

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.

Recent Accounting Pronouncements

For additional information, please read *Note 2, 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 March 31, 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 March 31, 2018, we had approximately $1,049.8 million of cash, cash equivalents and investments, comprised of $557.2 million of cash and cash equivalents, $491.8 million of short-term investments and $0.8 million restricted cash and investments. Our cash equivalents consist of commercial paper. 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 March 31, 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 March 31, 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 March 31, 2018, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarter ended March 31, 2018, we implemented a new enterprise resource planning (“ERP”) system which is expected to improve the efficiency of certain financial and related transaction processes. This system provides functionality to effectively support our expanding global operations. The implementation has resulted in certain business and operational changes. In connection with the implementation, we updated processes that are part of our internal control over financial reporting to accommodate related changes to our accounting procedures and business processes. Additionally, we implemented certain internal controls in connection with our adoption of ASC Topic 606. Otherwise, during the quarterly period ended March 31, 2018, there were no other 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. 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. 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. 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. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and /or substantial termination penalties, which could have a material adverse effect on our business prior to and after commercialization.

The third parties we use in the manufacturing process for EXONDYS 51 and our product candidates may fail to comply with cGMP regulations.

Our contract manufacturers are required to produce our materials, APIs and drug products under cGMP. We and our contract manufacturers are subject to periodic inspections by the FDA, 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, 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. 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, 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. 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.

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 May 3, 2018, the CHMP of the EMA has recently rendered a negative trend vote of our MAA for eteplirsen following our oral explanation, making a positive CHMP opinion unlikely. Although we plan to seek a re-examination and request that a SAG be called to provide expert guidance and insight into DMD, we may not be granted a re-examination, a SAG may not be convened, and even if a re-examination and a related SAG are granted, the CHMP may render a negative opinion.

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 preclinical 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 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). In addition, 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.

The remainder of our product candidates are in discovery or early stages of development. These product candidates will require significant further development, financial resources and personnel to develop into commercially viable products and obtain regulatory approval, if at all. Currently, our exon 45-skipping product candidate, the exon 53-skipping product candidate we are developing with the SKIP-NMD consortium, and the PPMO exon 51 product candidate, each for DMD, are in active clinical development. In addition, both the micro-dystrophin gene therapy program and the Galgt2 gene therapy program of Nationwide Children's Hospital, with whom we are collaborating, have entered into the clinic. Our other product candidates are in discovery, pre-clinical development or inactive. Given the FDA approval of EXONDYS 51, we expect that much of our effort and many of our expenditures over the next several years will be devoted to clinical development and regulatory activities associated with EXONDYS 51 and other exon-skipping candidates as part of our larger follow-on exon strategy in DMD, our other disease candidates, our proprietary chemistry, and other potential therapeutic areas that provide long-term market opportunities. We may be delayed, restricted, or unable to further develop our active and other product candidates or successfully obtain approvals needed to market them. Although EXONDYS 51 was approved under accelerated approval by the FDA in the U.S., we may not be able to obtain an approval of EXONDYS 51 in the EU.

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

Our RNA-targeted platform, utilizing proprietary PMO-based technology, has only been incorporated into one therapeutic commercial product to date, EXONDYS 51, however, our confirmatory trials for EXONDYS 51 must verify and describe the clinical benefits in order for EXONDYS 51 to remain approved in the U.S. 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 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, we cannot provide assurances that data from our studies with respect to EXONDYS 51, golodirsen, casimersen and gene therapy 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. 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.

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

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

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

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

We have historically relied on U.S. government contracts and awards to fund and support certain development programs. 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 contract research organizations ("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 $30.7 million for the three months ended March 31, 2018. Our accumulated deficit was $1.3 billion as of March 31, 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 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) 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), 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 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 JapanThese 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) 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) 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) 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 twenty seven 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 March 31, 2018, there were approximately 65.5 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 March 31, 2018, there were approximately 1.5 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan, approximately 0.2 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan and approximately 0.4 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan. We may issue additional common stock and warrants from time to time to finance our operations. We may also issue additional shares to fund potential acquisitions or in connection with additional stock options or other equity awards granted to our employees, officers, directors and consultants under our Amended and Restated 2011 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. The issuance of additional shares of common stock or warrants to purchase common stock and the perception that such issuances may occur or exercise of outstanding warrants or options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

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.

Seventh Amendment to a Lease Agreement between the Company and ARE-MA Region No. 38, LLC

On April 27, 2018 the Company and ARE-MA Region No. 38, LLC (the "Landlord") entered into a seventh amendment to the lease agreement dated June 25, 2013, as amended (the "Amendment" and the "Lease Agreement", respectively). Among other things, the Amendment extends the term of the Lease Agreement through September 30, 2025 and expands the size of the rentable square feet of laboratory and office space in a building located at 215 First Street, Cambridge, Massachusetts (the "Building") by adding approximately 63,698 rentable square feet of office space (the "Expansion Premises").

Under the terms of the Amendment, the Landlord will deliver certain parts of the Expansion Premises on July 1, 2018, other parts on October 1, 2018, and must use reasonable efforts to deliver the remaining parts (the "Seventh Expansion Premises") on or before November 1, 2018. The Company will commence paying rent with respect to each part of the Expansion Premises upon delivery of the relevant part, except that with respect to the Seventh Expansion Premises, the Company will commence paying rent 3 months following delivery.

Although the Amendment does not provide the Company with a termination option, it provides the Company with two consecutive rights to extend the term of the Lease Agreement for five years each, provided that the first extension must be with respect to not less than 75% of the entire then-existing premises being leased by the Company and the second extension must be with respect to not less than 100% of the entire then-existing premises being leased by the Company. Each extension will be at the Market Rate, as defined in the Amendment, and otherwise on the same terms and conditions as the initial term under the Amendment. The Company also has an expansion right to include additional space in the Building under the terms and conditions specified in the Amendment.

In addition to operating expenses and certain other additional expenses set forth in the Amendment, the Company will pay a blended average base rent of approximately $57.42 per square foot over a 7-year period.

The foregoing summary does not purport to be complete and is subject to, and qualified in its entirety by reference to, the complete copy of the Amendment that is filed as Exhibit 10.4 to this Quarterly Report on Form 10-Q.

Warrant to Purchase Common Stock of Myonexus Therapeutics, Inc.

On May 3, 2018, the Company purchased from Myonexus Therapeutics, Inc., a privately-held Delaware corporation ("Myonexus"), a warrant to purchase common stock of Myonexus (the "Warrant"), which, in combination with amendments to the Myonexus certificate of incorporation, provides the Company with an exclusive option (the "Option") to acquire Myonexus by making an option exercise payment to Myonexus plus contingent payments, if earned.

The Company may exercise the Option at any time prior to the 60th day after Myonexus completes 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 Myonexus' Phase 1/2A clinical trial of MYO-101 and provides the Company with the study's final biopsy results, subject to certain conditions. If the Company does not exercise the Option by such time, the Warrant provides that the Option will terminate. However, the Company has no obligation to exercise the Option and may also terminate the Warrant at any time for any reason prior to the option exercise deadline.

In consideration for the Warrant, the Company will pay Myonexus a $60 million upfront payment. If certain development milestones relating to Myonexus' product candidates are met prior to termination of the Warrant, the Company will pay Myonexus milestone payments up to an aggregate of $45 million if all milestones are met. If the Company exercises the Option, in addition to

their pro rata share of the exercise payment, Myonexus' former shareholders will also be entitled to receive their pro rata share of certain contingent payments upon achievement of a threshold amount of net sales of Myonexus products or the receipt and subsequent sale of a priority review voucher with respect to a Myonexus product.

The foregoing summary does not purport to be complete and is subject to, and qualified in its entirety by reference to, the complete copy of the Warrant, dated May 3, 2018, a copy of which will be filed with the Securities and Exchange Commission as an exhibit to the Quarterly Report on Form 10-Q for the quarter ending June 30, 2018.

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	
10.1†	General Release and Amendment to Separation Agreement between Sarepta Therapeutics, Inc. and Dr. Catherine Stehman-Breen dated April 12, 2018					X
10.2*	Sponsored Research Agreement between AVI BioPharma, Inc. and Charley's Fund, Inc., effective October 12, 2007					X
10.3*	First Amendment to Sponsored Research Agreement between AVI BioPharma, Inc. and Charley's Fund, Inc. dated June 2, 2009					X
10.4	Seventh Amendment to a Lease Agreement between the Company and ARE-MA Region No. 38, LLC dated April 27, 2018					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.

* This exhibit was previously filed with certain information redacted, and is being re-filed as an exhibit hereto in unredacted form.

** 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: May 3, 2018

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

Date: May 3, 2018

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

Exhibit 10.1

GENERAL RELEASE AND AMENDMENT TO SEPARATION AGREEMENT

GENERAL RELEASE AND AMENDMENT TO SEPARATION AGREEMENT (the "Release/Amendment"), by Dr. Catherine Stehman-Breen, M.D., M.S. (the "Executive") in favor of **Sarepta Therapeutics, Inc.** (the "Company") and the Company Releasees (as hereinafter defined), dated as of December 15, 2017.

Capitalized terms used herein but not specifically defined shall have the meanings set forth in the letter agreement between Executive and the Company, dated as of September 26, 2017 (the "Severance 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 below in accordance with the Severance Agreement, subject to the terms and conditions set forth therein.

NOW, THEREFORE, in consideration of the covenants and agreements hereinafter set forth, the parties agree as follows:

1. Separation Benefits. In connection with the termination of Executive's employment by the Company, the Company has agreed to provide Executive with the following payments and benefits, all as set forth in the Severance Agreement and subject to the terms and conditions set forth herein, including Executive's timely delivery and failure to revoke this Release/Amendment and subject to Executive's compliance with the terms and conditions of the Severance Agreement, including Sections 3, 4, 5, 6 and 7 of the Severance Agreement, as modified by Section 9 of this Release/Amendment:

(a) An aggregate total payment of $405,000.18, representing the amount set forth in Section 1(a)(i)(x) of the Severance Agreement, to be paid in substantially equal installments in accordance with the Company's regular payroll policies over a period of 12 months following termination;

(b) A lump sum target bonus of $324,000.14, representing the amount set forth in Section 1(a)(i)(y) of the Severance Agreement plus an amount equal to Executive's 2017 target bonus;

(c) A one-time payment of $924.49 (for December 15, 2017 through December 31, 2017) and subsequent monthly payments of $2,231.68, representing the amount set forth in Section 1(a)(ii) of the Severance Agreement, for the period beginning on December 15, 2017 and ending on the earlier of (x) 12 months following the date of such termination and (y) the date Executive becomes eligible for group health insurance coverage through a new employer, subject to the requirements set forth in Section 1(a)(ii) of the Severance Agreement;

(d) Treatment of stock options:

(i) Twenty-five percent (25%) of the stock option award listed in Annex C of the Severance Agreement shall vest and be exercisable on April 30, 2018; and

(ii) Commencing on May 1, 2018, continued monthly vesting on the 30th day of each month of the stock option award listed in Annex C of the Severance Agreement at the rate of 1/48th of the total shares underlining such stock option for 12 additional months, notwithstanding the vesting schedule that would have applied had Executive remained continuously employed by the Company during such 12- month period. Any portion of the stock option award that is not vested by the dates set forth herein shall be forfeited and cancelled in its entirety on such applicable date.

Each such payment or benefit shall be paid at the time set forth in Section 1(b) of the Severance Agreement and shall be subject to the terms and conditions of such section. Further, for the stock option award that vests under Section 1(d) of this Release/Amendment, following Executive's termination, Executive shall be entitled to exercise any vested portion of such stock option award following vesting and until the end of the 90-day period following the end of the additional 12- month period set forth in subsection 1(d)(ii) of this Release/Amendment.

2. General Release by Executive. 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 Severance Agreement and/or Executive's Change in Control and Severance Agreement dated February 13, 2017, 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 Release/Amendment, (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 of 1974, 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 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.

3. Surviving Claims. Notwithstanding anything herein to the contrary, this Release/Amendment 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 Severance Agreement or this Release/Amendment, 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, or (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.

4. 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.

5. Acknowledgements by Executive. Executive acknowledges and agrees that Executive has read this Release/Amendment in its entirety and that this Release/Amendment is a general release of all known and unknown rights and Claims, including, without limitation, of rights and Claims arising under ADEA. This Release/Amendment 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/Amendment does not release, waive or discharge any rights or claims that may arise for actions or omissions after the date of this Release/Amendment;

b. Executive is entering into this Release/Amendment 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/Amendment, to consult with an attorney before executing this Release/Amendment, and Executive has consulted with counsel of Executive's choice concerning the terms and conditions of this Release/Amendment;

d. Executive has been advised, and is being advised by this Release/Amendment, that Executive has forty-five (45) days within which to consider this Release/Amendment, and that 45-day period has been extended by mutual agreement of the parties; and

e. Executive is aware that this Release/Amendment shall become null and void if Executive revokes Executive's agreement to this Release/Amendment within seven (7) days following the date of execution of this Release/Amendment. Executive may revoke this Release/Amendment 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/Amendment no later than 5:00 p.m. Eastern Time on the seventh (7th) full day following the date of execution of this Release/Amendment (the "Effective Date").

6. Additional Agreements. Nothing in this Release/Amendment 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/Amendment 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 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, Executive acknowledges and understands that under the Federal Defend Trade Secrets Act of 2016, 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 Executive's attorney in relation to a lawsuit for retaliation against 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.

7. Confidentiality Agreement. Executive acknowledges and agrees that she is subject to the terms and conditions of the Confidentiality Proprietary Rights and Non-Disclosure Agreement with the Company, dated as of February 13, 2017, and that she will continue to comply with, and abide by, such agreement.

8. Restrictive Covenants. Executive acknowledges and agrees that she is subject to the terms and conditions of Sections 3, 4, 5, 6 and 7 of the Severance Agreement, with the following modifications:

The second through fourth sentences of Section 5 of the Severance Agreement regarding the non-competition covenant shall be replaced with the following language:

"Accordingly, during your employment hereunder and for the Restriction Period, you agree that you 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: (i) any person, firm, corporation or other entity, in whatever form, engaged in the research, development or sale of Duchenne Muscular Dystrophy treatments that compete with the Company or any of its subsidiaries or affiliates; or (ii) any of the following companies: Wave Life Sciences Ltd., Nippon Shinyaku Co., Ltd., Exonics Therapeutics, Daiichi Sankyo and Solid Biosciences Inc., any of their subsidiaries or affiliates, or any of their successors. Notwithstanding the foregoing, nothing herein shall prohibit you 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 you have no active participation in the business of such corporation. Provided further, the provisions of subsection (i) of this Section shall not be violated by you being employed with a subsidiary, division or unit of any such entity described in subsection (i) so long as you and such subsidiary, division or unit do not engage in the research, development or sale of Duchenne Muscular Dystrophy treatments in competition with the Company or any of its subsidiaries or affiliates."

9. Company Non-Disparagement. For a period of one (1) year following the termination of Executive' employment, the Company shall advise in writing its officers and

directors to not publicly or privately, disparage, criticize or defame Executive. Nothing shall prevent Executive or the Company's officers and directors from making any truthful statement (i) to the extent necessary in connection with any litigation, arbitration or mediation involving this Release/Amendment or the Severance Agreement, including, but not limited to, the enforcement of this Release/Amendment or (ii) to the extent required by law or by any court, arbitrator, mediator or administrative or legislative body (including any committee thereof) with apparent jurisdiction or authority to order or require such person to disclose or make accessible such information.

10. Cooperation. Executive acknowledges and agrees that she is subject to the terms and conditions of Section 7 of the Severance Agreement, with the following modifications:

The following language shall be added to the end of the paragraph:

"The Company agrees to pay Executive for the time she spends complying with this paragraph at the hourly rate of $195.00 (based upon her base salary at the time of termination)."

11. No Debts Owed by Executive to Company. The Company acknowledges and agrees that as of the date of this Agreement, Executive owes no money or other debt to Company, including but not limited to any funds paid to Executive in connection with her relocation.

12. Amendment. No provision of this Release/Amendment 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.

13. Interpretation. To the extent that any inconsistencies exist between this Agreement and the Separation Agreement, the terms of this Agreement shall control.

IN WITNESS WHEREOF, Executive has signed this Release/Amendment on the date set forth below.

EXECUTIVE

By: /s/ Catherine Stehman-Breen, M.D.
Name: Catherine Stehman-Breen, M.D.
Date: 4/12/2018

SAREPTA THERAPEUTICS, INC.

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

Exhibit 10.2

SPONSORED RESEARCH AGREEMENT

THIS SPONSORED RESEARCH AGREEMENT (this "Agreement"), effective the 12th day of October, 2007 (the "Effective Date"), is entered into by and between AVI BIOPHARMA, INC., an Oregon Corporation, with principal offices located at One SW Columbia, Suite 1105, Portland, Oregon 97258 ("Company"), and CHARLEY'S FUND, INC., a 501(c)(3) tax-exempt public non-profit organization with a mailing address of P.O. Box 297, South Egremont, MA, 01258 (the "Sponsor").

W I T N E S S E T H:

WHEREAS, the Sponsor wishes to promote scientific research leading to exon skipping therapeutics related to Duchenne muscular dystrophy; and

WHEREAS, the Company has developed a proprietary antisense chemistry and has certain employees who possess knowledge, know-how and experience in substantive fields relating to such research and commercialization efforts;

WHEREAS, the Company has a cooperative development and license agreement with Ercole Biotech, Inc., and cross licensing rights to intellectual property important for the freedom to operate;

WHEREAS, the Sponsor is willing to fund such research by the Company, with the objective, as set forth herein, leading to an Investigational New Drug (IND) filing with the Food and Drug Administration; and

WHEREAS, it is the intent of the Sponsor and the Company to complete the research to identify a viable candidate and to demonstrate the safety and efficacy of the putative therapeutic used for exon skipping, sufficiently to file an IND and proceed to human clinical trials required for regulatory approval of said therapeutic,

NOW, THEREFORE, in consideration of the premises herein contained, and for other good and valuable consideration, the parties agree as follows:

1. Definitions

For purposes of this Agreement, the following definitions apply:

1.1 "Affiliates" shall mean any corporation or other entity that controls, is controlled by, or is under common control with, a party. A corporation or other entity shall be regarded as in control of another corporation or entity if it owns or directly or indirectly controls more than 50% of the voting securities or other ownership interest of the other corporation or entity, or if it possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of the corporation or other entity.

1.2 "Agreement Period" shall mean the period commencing on the Effective Date of this Agreement and ending upon completion of the Research Project.

1.3 "Company Inventions" shall have the meaning provided in Section 8.1 hereof.

1.4 "Confidential Information" shall have the meaning provided in Section 6.1 hereof.

1.5 "FDA" shall mean the United States Food and Drug Administration.

1.6 "Field" shall mean the treatment or prevention of Duchenne Muscular Dystrophy or any other muscular dystrophy.

1.7 "Invention" shall have the meaning provided in Section 8.1 hereof.

1.8 "Investigators" shall have the meaning provided in section 2.1 hereof.

1.9 "Joint Inventions" shall have the meaning provided in Section 8.1 hereof.

1.10 "Major Market" shall mean any one of the following:

(a) The United States;

(b) Japan; or

(c) Any of the following five (5) European countries: Germany, The United Kingdom, France, Italy, or Netherlands.

1.11 "Net Sales" means the gross amount invoiced for sales of Research Products by Sponsor, its affiliates, and sublicensees, to an independent third party in an arms-length transaction, less:

(a) Trade, quantity and cash discounts allowed;

(b) Discounts, refunds, rebates, chargebacks, retroactive price adjustments, and any other allowances which effectively reduce the net selling price;

(c) Credits for actual Research Product returns;

(d) Any tax imposed on the production, sale, delivery or use of the Research Product, including, without limitation, sales, use, excise or value added taxes.

1.12 "Option" shall have the meaning provided in Section 9.2 hereof.

1.13 "Option Term" shall have the meaning provided in Section 9.2 hereof.

1.14 "Project Funds" shall have the meaning provided in Section 4.1 hereof.

1.15 "Project Team" shall have the meaning provided in Section 2.1 hereof.

1.16 "Research Product" shall have the meaning provided for in Section 4.3.6 hereof

1.17 "Research Project" shall mean the research described in the Study Protocol hereof.

1.18 "Results" shall have the meaning provided in Section 3.4 hereof.

1.19 "Sponsor Inventions" shall have the meaning provided in Section 8.1 hereof.

1.20 "Study Protocol" shall mean the protocol set forth in Appendix A with attached Gantt chart hereto, which is incorporated herein by reference and made a part hereof as if fully set forth herein, as such protocol may be modified from time to time by mutual written agreement of the Company and the Sponsor.

2. Research

2.1 The Principal Investigator for the Research Project shall be Dr. Patrick Iversen. The Principal Investigator shall be responsible for directing and overseeing the conduct of the Research Project using appropriately qualified collaborating investigators, including Dr. Ryszard Kole, CSO, Ercole Biotech, Dr. Stephen Wilton, University of Western Australia, and Dr. Qi Lu, Carolina's Healthcare Foundation, and scientists and research technicians who are under the Principal Investigator's direction and control and are employed by the Company (collectively, the Principal Investigator, the collaborating investigators, and the Company employees working on the Research Project constitute the "Project Team").

2.2 Subject to the terms and conditions of this Agreement, the Company and the Principal Investigator shall perform the Research Project in accordance with the Study Protocol. No change to the Study Protocol shall be effective without the prior written consent of the Sponsor. The Company and the Principal Investigator shall use reasonable efforts to distinguish the research performed under this Agreement from all other work the Principal Investigator performs for other purposes and shall keep records pertaining to such other work separately from the records to be maintained pursuant to Section 2.7 to the extent practicable.

2.3 The Company shall provide the Sponsor with written evidence of approval of the Research Project by the responsible body within the Company, if such approval is necessary, and with copies of any documents used in the conduct of the Research Project, including, but not limited to, all documentation required by the Study Protocol.

2.4 The Company shall provide the support necessary for the Project Team to complete the Research Project, which support shall include, but is not limited to, human resources, space, dedicated research time, and computing, laboratory, and all other equipment, all in accordance with the Study Protocol attached as Appendix A.

2.5 The Company shall also accept and administer the Project Funds. The Company's use of the Project Funds shall be strictly for purposes of the Research Project and shall be subject to the terms and conditions set forth in APPENDIX B, which is incorporated herein by reference and made part of this Agreement. NO PART OF THE PROJECT FUNDS SHALL BE USED FOR INDIRECT EXPENSES OF THE PROJECT TEAM OR THE Company. Except as specified in the Study Protocol relating to work to be conducted by collaborating investigators, no part of the Project Funds shall be transferred to another organization, whether or not the Principal Investigator or any other member of the Project Team becomes associated with that other

organization unless the prior written consent of the Sponsor is obtained by the Company. The Company shall be required to repay to the Sponsor any part of the Project Funds used in contravention of the express terms of this Agreement.

2.6 The Company shall ensure that the Research Project shall be conducted in strict compliance with any applicable federal, state, or local laws, regulations, or guidelines pertaining to good research practices and/or good laboratory practices.

2.7 The Company shall keep accurate and complete financial and scientific records relating to the Research Project and will make such records reasonably available to the Sponsor for review and/or copying during normal business hours.

2.8 The Company shall promptly advise the Sponsor of any changes in the senior personnel comprising the Project Team. If, for any reason, the Principal Investigator (i) ceases to be associated with the Company, (ii) becomes debarred or receives notice of an action or threat of an action with respect to debarment under the provisions of the Generic Drug Enforcement Act of 1992, 21 U.S.C. Section 306(a) and (b), or (iii) otherwise becomes unavailable to work on the Research Project, the Company shall promptly so notify the Sponsor in writing and will propose a qualified replacement scientist at the Company whose appointment as Principal Investigator shall be subject to the approval of Sponsor. The Company shall consult with and reasonably consider and take into account the Sponsor's view with respect to the replacement of the Principal Investigator, provided that, in the case of a proposed replacement chosen by the Company and who is on the Advisory Committee, the Sponsor agrees that it will give its approval to such replacement for the Principal Investigator.

3. Reports to the Sponsor

3.1 During the Agreement Period, the Sponsor may meet with the Principal Investigator from time to time to discuss the planning and progress of the Research Project. An Advisory Committee made up of three members or advisors from the Company, three members or advisors from the Sponsor, a member from Ercole Biotech and an external collaborator will meet once per quarter to review progress of the Research Project. The Company will have final decision-making authority on all drug development, strategic, and other decisions.

3.2 During the Agreement Period and for three (3) years thereafter, the Company shall make available to the Sponsor copies of all data and other information generated pursuant to this Agreement including, without limitation, all raw data obtained as a result of studies conducted in the course of the Research Project and all experimental procedures developed under the Research Project.

3.3 At least every three (3) months during the conduct of the Research Project, the Company, in coordination with the Principal Investigator, shall provide the Sponsor with an interim written progress report concerning the Research Project.

3.4 A final written report setting forth the results achieved under and pursuant to the Research Project and recommendations for next actions shall be submitted by the Company to the Sponsor within ninety (90) days of completion or earlier termination of the research that is the subject of

this Agreement. Such final report shall include a complete summary of the research carried out and detailed experimental results of the research protocols performed in the course of the Research Project (collectively, the "Results").

3.5 Each written progress report to the Sponsor, including the final report, shall be accompanied by a financial statement from the Company describing in reasonable detail the disposition to date of the Project Funds.

3.6 During the Agreement Period and for five (5) years thereafter, authorized employees and agents of the Sponsor or of the FDA shall have access to the Company and its personnel and records relating to the Research Project for the purpose of determining compliance with this Agreement and the Study Protocol and federal, state, and local laws and regulations and any applicable guidelines for the conduct of research. Such access by employees and agents of the Sponsor shall be on reasonable notice and during normal business hours, and individuals conducting such visits shall be bound by appropriate confidentiality agreements with the Company.

4. Payments and Repayment Rights

4.1 Subject to the terms and conditions of this Agreement including the repayment rights provided for in Section 4.3, the Sponsor shall pay the Company a total amount of Two Million Four Hundred and Fifty-two Thousand Dollars ($2,452,000.00) which amount 1s inclusive of all direct costs of Research Project activities (the "Project Funds") as follows:

Four Hundred Thousand Dollars ($400,000.00) shall be paid within ten (10) days of the parties' execution of this Agreement; Nine Hundred Thousand Dollars ($900,000.00) shall be paid three (3) months from the Effective Date of this Agreement subject to Sponsor's receipt of the first progress report demonstrating completion of that research component of the Research Project; Seven Hundred Thousand Dollars ($700,000.00) shall be paid six (6) months from the Effective Date of this Agreement subject to the Sponsor's receipt of the second progress report demonstrating completion of that research component of the Research Project; and Four Hundred and Fifty-Two Thousand Dollars ($452,000.00) shall be paid nine (9) months from the Effective Date of this Agreement subject to the Sponsor's receipt of the third progress report demonstrating completion of that research component of the Research Project. The Sponsor shall not be obligated to make any payments to the Company in addition to those set forth in this Section 4.1 unless the parties otherwise mutually agree in writing.

4.2 The Company shall provide to the Sponsor all information necessary to make the payments described above, including, but not limited to, the name of the payee, its tax identification number, and the name and address of the contact person to whom payments should be sent.

4.3 The Company and the Sponsor agree to the following commercial terms with regard to the development and commercialization of a Research Product:

4.3.1 The Company shall make a lump sum payment to the Sponsor of Eight Hundred and Seventeen Thousand Three Hundred and Thirty Four Dollars ($817,334.00)

(the "First Payment") within thirty (30) days after the end of the fiscal quarter during which the first commercial sale into a Major Market of the Research Product occurs.

4.3.2 The Company shall make a lump sum payment to the Sponsor of Eight Hundred and Seventeen Thousand Three Hundred and Thirty Three Dollars ($817,333.00) (the "Second Payment") within thirty days (30) after the end of the fiscal quarter during which the first anniversary of the first commercial sale into a Major Market of the Research Product occurs.

4.3.3 The Company shall make a lump sum payment to the Sponsor of Eight Hundred and Seventeen Thousand Three Hundred and Thirty Three Dollars ($817,333.00) (the "Third Payment") within thirty days (30) after the end of the fiscal quarter during which the second anniversary of the first commercial sale into a Major Market of the Research Product occurs.

4.3.4 In the event the Company or one of its Affiliates enters into any sort of partnership (a license agreement, research and development agreement, collaboration or similar arrangement) with a corporate partner that includes the right to sell, distribute promote or market the Research Product or any of the underlying intellectual property and

(i) if, prior to the second anniversary of the first commercial sale of a Research Product in a Major Market country, the corporate partner agrees to pay an upfront cash license fee or similar payment which is earned upon signing, the Company or its Affiliates shall pay to the Sponsor, within thirty (30) days of Company's receipt of such payment from the corporate partner, 15% of the cash received as an upfront fee or the total Project Funds, less any amount already repaid to the Sponsor by the Company, whichever is less.

(ii) and if, thereunder, the Company is entitled to development milestone payments, the Company or its Affiliates shall, within thirty (30) days of receipt of any such payments, make repayments to the Sponsor in the amount of 15% of each individual milestone payment specifically related to the progress for the development of a Research Product, limited to 50% of the Project Funds at each of such milestone payment, until the Project Funds amount is repaid in full.

4.3.5 Without limiting the foregoing, in the event that the full amount of the Project Funds have not been repaid to the Sponsor at first commercial sale into a Major Market of the Research Product via the payment mechanisms of Section 4.3.4, the Company shall make payments to the Sponsor as provided for in Sections 4.3.1, 4.3.2, and 4.3.3..

4.3.6 "Research Product" shall mean any product containing any molecular candidate arising or derived from the research funded hereunder which is developed as a human therapeutic agent for skipping exon 50 in the indication Duchenne Muscular Dystrophy.

4.3.7 Repayments to the Sponsor under all mechanisms in this Article 4 shall not exceed the total Project Funds as provided for in Section 4.1.

5. Right of the Company to Seek Additional Funding

5.1 The Sponsor strongly encourages the Company, through the efforts of the Principal Investigator, to seek additional funding for the laboratories of the Investigators from the Federal government or other sponsors of research and acknowledges that such additional sponsors may retain rights in and to such funded research.

6. Confidentiality

6.1 The Sponsor and the Company acknowledge that each party may receive confidential technical and business information of the other party ("Confidential Information") during the Agreement Period. Each party hereto agrees that, during the Agreement Period and for a five (5) year period thereafter, that it will maintain in strict confidence, and will not disclose to any third party, any Confidential Information of the other party, whether in oral, written, graphic or electronic form. Each party hereto agrees (i) not to use Confidential Information of the other party except for purpose of conducting Research Project activities in accordance with the Study Protocol or for such other purposes consistent with the intent and terms of this Agreement and (ii) not to disclose Confidential Information of the other party to third parties without the express written permission of the other party, except that (a) each party shall not be prevented from disclosing Confidential Information to its employees, officers, independent contractors and Affiliates requiring access thereto for the purposes of this Agreement provided each such employee, officer, independent contractor or Affiliate is bound by an agreement regarding confidentiality and non-use at least as restrictive as the obligations in this Article 6, and (b) such information may be disclosed insofar as such disclosure is necessary to allow either the Company or the Sponsor, as the case may be, (A) to defend itself against litigation, (B) to file and prosecute patent applications on any Invention in accordance with Article 8 hereof, or (C) to comply with judicial decree, government action or applicable law or regulation, provided that the party shall give prior written notice to the other party so that the other party may attempt to obtain a protective order requiring that the Confidential Information be disclosed only to the extent required by such order, law or regulation, and that it be used only for the purposes for which the decree, action, law or regulation requires such disclosure to be made. The parties agree that no advance notice to the Sponsor is required for AVI’s compliance with its reporting requirements to the Securities and Exchange Commission (SEC). The parties will take all steps necessary to ensure that its employees, officers, independent contractors, and Affiliates comply with the terms and conditions of this Agreement. Notwithstanding the foregoing, such obligation of confidentiality shall not apply to information that the receiving party can establish by competent evidence:

(i) at the time of disclosure is in the public domain;

(ii) has come into the public domain through no fault of the receiving party or its employees and agents;

(iii) was known to the receiving party prior to its disclosure by the disclosing party, as evidenced by the receiving party's written records; or

(iv) is disclosed to the receiving party, without restriction on disclosure, by a third party that is not under an obligation of non-disclosure to the disclosing party.

6.2 The Company (including, for purposes of this Section 6.2, the Principal Investigator) shall have the right, and is encouraged, to publish or present the Results of the Research Project, provided the Sponsor has the opportunity to review and comment on any proposed manuscripts or the substance of any presentations describing said Research Project or Results at least thirty (30) days prior to their submission to a third party for publication or review. In the event that the rights to the Research Product have been licensed to Sponsor pursuant to Article 9, then the positions of the Parties with respect to the provisions of this Section 6.2 shall be reversed. The reviewing party shall review any draft and give its comments to the publishing party promptly. The publishing party shall comply with the reviewing party's request to delete references to the reviewing party's Confidential Information in any such publication and the publishing party agrees to withhold publication an additional thirty (30) days to permit the reviewing party to obtain patent or other intellectual property protection, if the reviewing party deems it necessary.

7. Use of the Other Party's Name; Public Statements

Each party agrees that it will not at any time during or following expiration or termination of this Agreement use the name of the other party or its employees or any other names, insignia, symbol(s), or logotypes associated with the other party or any variant or variants thereof orally or in any literature, advertising, or other materials without the prior written consent of the other party except for right to publish set forth in Section 6.2 and Company's compliance with its reporting requirements to the SEC, which consent may be withheld at the other party's sole discretion. Notwithstanding the foregoing, the Company agrees that the Sponsor may use the names of the Company, the Principal Investigator and his collaborators in connection with generally publicizing on its website, in press releases or in other publications of the Sponsor provided that such usages are limited to identifying the Company and/or the Principal Investigators and briefly describing the nature of the Research Project and the Sponsor agrees that Company may use Sponsor's name in connection with any board or investor presentation, or press release related thereto, or as may be requested by any funding entity, governmental entity, or academic publisher, or as required by law. Prior to publicizing, both parties agree to give the other party an opportunity to review press releases using the other's name and discussing the work involved in the project.

8. Ownership and Patents

8.1 Company shall own all data obtained in the Research Project, research protocols related to the Research Project, and Results, and shall have the right to submit all such information to support regulatory filings related to the Research Product and other products that may be developed by the Company.

8.2 Ownership of any discovery, invention, method, process or other know-how made, conceived or first reduced to practice in the performance of the Research Project during the project period by the Company and its affiliates, the Principal Investigator and/or the Sponsor and all intellectual property arising therefrom (collectively, "Inventions") shall be determined as follows: All Inventions conceived or reduced to practice during the project period solely by employees, agents or consultants of the Company, including, without limitation, the Principal Investigator ("Company Inventions") shall be owned solely by the Company. All Inventions conceived or reduced to practice during the project period solely by employees, agents or consultants of the

Sponsor ("Sponsor Inventions") shall be owned solely by the Sponsor. All Inventions conceived or reduced to practice jointly during the project period by employees, agents or consultants of the Company, on the one hand, and employees, agents or consultants of the Sponsor, on the other hand ("Joint Inventions"), shall vest according to U.S. patent law. The Company represents and warrants that all Company employees and other individuals or entities performing any part of the Research Project are obligated to assign to the Company all inventions and intellectual property rights that are necessary to enable the Company to grant the Sponsor all rights the Company purports to grant under this Agreement.

8.3 As soon as the Company reasonably believes a Company Invention or Joint Invention has been conceived or reduced to practice hereunder (and in any event within a reasonable time after its disclosure to the Company Technology Office), the Company shall disclose such invention in writing to the Sponsor in sufficient detail to allow the Sponsor to evaluate its significance.

8.4 The Company shall have the first right to prosecute any patent application(s) covering any Company Inventions. Within a reasonable time after disclosure of any such Company Invention to the Sponsor, the Company shall notify the Sponsor in writing if it intends to pursue patent protection for such Company Invention. If the Company elects to pursue patent protection, it shall promptly prepare, file and prosecute any U.S. or foreign application(s) to protect such Company Invention. The Company shall bear all expenses in connection with such preparation, filing, prosecution and maintenance of U.S. and foreign patent applications. For such Company elected patent applications, the Company shall be responsible for making decisions regarding the scope and content of such patent application(s) and the prosecution thereof. The Sponsor shall be responsible for the costs of patent filing and prosecution for Sponsor Inventions or if the Sponsor requests that the Company files a patent application. The Company and Sponsor shall each make reasonable efforts to keep the other advised as to all developments with respect to such application(s).

8.5 For Joint Inventions, the Company and Sponsor will negotiate in good faith, at the time of disclosure, the management and prosecution of the invention including any cost related thereto.

8.6 Sponsor grants Company an exclusive, worldwide, fully paid-up, royalty-free license under Sponsor's interest in any Joint Inventions and to Sponsor Inventions to make, use and sell Research Products.

9. <u>Granting of Exclusive License</u>

9.1 The parties acknowledge and agree that they intend to use their reasonable best efforts to complete the Research Project as described in the Study Protocol. In the event the Company and its Affiliates and partners elect to discontinue to pursue the development and/or commercialization of a Research Product for reasons other than safety and efficacy, the Company and its Affiliates, at the request of the Sponsor, hereby grants the Sponsor the exclusive royalty-bearing, fully paid up, worldwide license or sublicense as the case may be, with the right to sublicense, to the Research Product, on terms consistent with the requirements of the Ercole Biotech-Isis Pharmaceuticals collaboration agreement and Ercole Biotech-AVI BioPharma collaboration agreement, under patents owned or licensed by the Company or its Affiliates to research, to develop, to use, to sell, to offer for sale, to distribute Research Products, to import, to export and to employ methods covered by any such patents or by Company Inventions or

Company's interest in Joint Inventions relating to the Research Product. In consideration for the exclusive license to use and sell the Research Product

9.1.1 if the Sponsor obtains a license to the Research Product while the Research Product is in the research or preclinical phase of the Research Project, a total royalty of 8% of Net Sales of Research Product shall be paid to the Company and its Affiliates by the Sponsor and its licensees.

9.1.2 If the Sponsor obtains a license to Research Product during or at the conclusion of Phase I clinical testing, a total royalty of 12% of Net Sales of Research Product shall be paid to the Company and its Affiliates by the Sponsor and its licensees.

9.1.3 If the Sponsor obtains a license to Research Product after the initiation of a Phase II clinical trial, a total royalty of 15% of Net Sales of Research Product shall be paid to the Company and its Affiliates by the Sponsor and its licensees.

9.2 The Company shall have the right of first refusal to manufacture research, clinical and commercial quantities of Research Product for the Sponsor or the Sponsor's commercial partner. Should the Company not exercise its first right to manufacture, the exclusive license granted to the Sponsor shall be expanded to include the right to develop to make, and to have made, Research Product and to employ methods covered by or incorporating Company Inventions or Company's interest in Joint Inventions which permit the commercialization of the Research Product to the worldwide market. The Company shall transfer the Research Product production process to a mutually agreeable third party contract manufacturer under an agreement that contains appropriate provisions for recovery of technology transfer costs and for limiting disclosure or other use of the Company's technology.

9.3 Except as expressly provided herein and including Article 9, nothing in this Agreement shall restrict either party's use, license or exploitation in any way of its interest in its own or any Joint Inventions.

10. Termination

10.1 This Agreement shall remain in effect for the Agreement Period unless extended by written agreement of the parties, or earlier terminated in accordance with this Article 10.

10.2 Either party may terminate this Agreement for any material breach of this Agreement by the other party if such breach is not cured within thirty (30) days after the breaching party receives written notice of such breach by the non-breaching party. Such termination shall be effective upon expiration of such thirty (30) day period.

10.3 Termination of this Agreement shall not affect the rights and obligations of the parties that shall have accrued prior to termination, including, without limitation, the confidentiality obligations set forth in this Agreement. In the event of any termination of this Agreement prior to expiration of the Agreement Period (other than termination by the Sponsor pursuant to Section 10.2), the Sponsor shall pay the reasonable costs incurred by the Company in winding down and terminating the Research Project, including the costs of the Research Project during the wind-down

period and all costs and non-cancelable commitments made prior to termination. After termination, the Company will submit a final report of all costs incurred and all funds received under this Agreement as set forth in Section 3.4. The report shall be accompanied with a check for any funds remaining which were paid to the Company under Section 4.1, if any. The provisions of Sections 2.7, 3.2, 3.4, 3.5, 3.6, 4.3 and 10.3 and Articles 6, 7, 8, 9, 12, 13, 16, 17, 18, 19, 20 and 21 shall survive termination or expiration of this Agreement.

11. Force Majeure

Neither party shall be responsible to the other for any failure or delay in performing any of its obligations under this Agreement if such delay or nonperformance is caused by strike, stoppage of labor, lockout or other labor trouble, fire, flood or other weather event, earthquake, accident, explosion, war, act of terrorism, act of God or act of the government of any country or of any local government or any other cause beyond the reasonable control of the defaulting party.

12. Liability and Warranty

Each party to this agreement agrees to indemnify the other party from damage to persons or property resulting from the negligence on the part of itself, its employees, its agents, or its officers. Neither party assumes any responsibility to the other party for the consequences of any act or omission of any person, firm or corporation not a party to this agreement.

The research results are preliminary in nature. Company makes no representations and extends no warranties of any kind, either expressed or implied, with regards to research results.

13. Independent Contractors

The Sponsor and the Company shall at all times act as independent parties, and nothing contained in this Agreement shall be construed or implied to create an agency or partnership. Neither party shall have the authority to contract or incur expenses on behalf of the other except as may be expressly authorized by separate written agreement between the parties. The Principal Investigator and members of the Project Team shall not be deemed to be employees of the Sponsor.

14. Other Employment

The Company warrants that the Principal Investigator is permitted to enter into this Agreement (but not to bind the Company) and that the terms and conditions hereof are consistent with the Principal Investigators' obligations to the Company.

15. Tax Status

The Company represents and certifies to the Sponsor that it is a publicly traded company.

16. Choice of Law

This Agreement shall be governed by and shall be construed in accordance with the laws of the Commonwealth of Massachusetts without regard to the conflicts of laws provisions thereof.

17. Severability

If any one or more of the provisions of this Agreement shall be held to be invalid, illegal or unenforceable, the validity, legality or enforceability of the remaining provisions of this Agreement shall not in any way be affected or impaired thereby.

18. Waiver

The failure of any party hereto to insist upon strict performance of any provisions of this Agreement or to exercise any right hereunder will not constitute a waiver of that provision or right.

19. Notices

Any notice or communication required or permitted to be given or made under this Agreement by one of the parties to the other shall be in writing and shall be deemed to have been sufficiently given or made for all purposes if such notice or communication is either emailed and its receipt is acknowledged by the recipient, or mailed by certified mail, postage prepaid, addressed to such other party at its respective address as follows:

If to the Sponsor: Attn: Benjamin D. Seckler, MD,
President Charley's Fund, Inc.
P.O. Box 297
South Egremont, MA 01258
Email:

If to the Company: Attn: Chief Executive Officer
AVI BioPharma, Inc.
One SW Columbia, Suite 1105,
Portland, Oregon 97258

20. Assignment

This Agreement may not be assigned by the Company without prior notice to Sponsor, except in connection with a merger, recapitalization, reorganization, consolidation, sale of securities, sale of assets or any transaction to an affiliate of the Company; provided, however, no such transaction shall relieve Company of its obligations or adversely affect Sponsor's rights hereunder. The Sponsor may assign this Agreement without the Company's consent (i) in connection with a merger, consolidation or sale of all or substantially all of Sponsor's assets or stock, or (ii) to an affiliate of the Sponsor. In addition, the Sponsor may assign all or any part of its rights under Articles 8 and 9 to any third party upon written notice to the Company.

21. Entirety

This Agreement represents the entire agreement of the parties, and it expressly supersedes all previous written and oral communications between the parties. Except as otherwise expressly provided in this Agreement, no amendment, alteration, or modification of this Agreement or any

Appendix attached hereto shall be valid unless executed in writing by authorized signatories of both parties.

IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be executed by their duly authorized representatives to be effective as of the Effective Date.

CHARLEY'S FUND, INC.	By: /s/ Benjamin D. Seckler Name: Benjamin D. Seckler, MD Title: President
AVI BIOPHARMA, INC.	By: /s/ K. Michael Forrest Name: K. Michael Forrest Title: Chief Executive Officer

APPENDIX A

Preclinical Proposal for Skipping Exon 50 for Duchenne Muscular Dystrophy

This proposal to Charley's Fund describes a research and preclinical development program to select and advance a compound to treat patients with Duchenne muscular dystrophy who could benefit by skipping exon 50 of the dystrophin gene. The research proposed includes a thorough, but rapid evaluation of enhancements to AVI's standard PMO chemistry that could improve drug potency and the targeting of the drug to critical tissues, including skeletal muscle and the heart. AVI believes that this evaluation will yield a clinical drug candidate with the highest likelihood of having meaningful therapeutic benefit.

I. Background:

The favorable characteristics of phosphorodiamidate morpholino oligomers (PMOs) include long term stability, simple dose regimens, and an outstanding safety profile, as follows:

1) long term stability- real time stability testing on PMOs stored for 2 years as lyophilized powder or as sterile saline solutions indicate less than 2% loss of active pharmaceutical ingredient (API).

2) PMOs have shown favorable pharmacokinetic properties leading to simple dose regimens. A phase I study with AVI-4065, targeting hepatitis C virus, was recently completed in healthy volunteers with subcutaneous dosing of 50, 100 and 300mg daily for 14 days. The fractional bioavailability based on AUC from this extravascular route appears to be greater than 1.0. The maximal plasma concentrations were predicted with precision from studies in rat and non-human primate. Finally, the apparent elimination half-life observed in plasma was approximately 10 hours but residence time in patients based on urinary clearance was nearly 11 days. We have conducted pharmacokinetic studies with four different PMOs in humans and have never detected a PMO metabolite.

3) The PMO technology has an outstanding safety profile. AVI has completed 17 clinical trials with four different PMO candidates in over 400 subjects and there have been no definitely drug related serious adverse events. It has been ethical and logical with these drugs to restrict volunteer populations to adults (viz., age of majority to 70 years) and not to children or infants. Subjects enrolled in these studies have been racially diverse and approximately half males and half females. Also, it should be noted that no female volunteers in AVI-sponsored clinical trials were pregnant or breast-feeding, and all women of child-bearing potential practiced acceptable birth control for the duration of active study surveillance.

AVI BioPharma is developing a PMO to skip exon 51 for treatment of Duchenne muscular dystrophy. Pharmacokinetic modeling based on animal data suggests that a dose of 100 mg administered subcutaneously once weekly should be effective. However, this will need to be

determined empirically in a dose-ranging clinical study. While we are optimistic that the exon 51 compound, AVI-4658, will be effective in this dose range, the possibility that significantly higher doses will be required to achieve efficacy leads us to search for more potent agents. We believe we can do this for exon 50 through the use of cell penetration enhancers, enhanced potency and improved tissue delivery. These measures have the potential to increase potency several fold. In addition, more potent agents result in less body burden of drug and hence better compliance and less likelihood of toxicity.

II. Applicable Work in Progress

A. Objective: Identify the Exon 50 sequence.

Analysis of PMOs in cell culture is well suited to identification of agents capable of inducing molecular events such as exon skipping. These studies provide valuable mechanistic information but are not effective predictors of potency or dose for *in vivo* activity. Hence, cell culture studies will be used to identify the sequence of the PMO for skipping exon 50. The current plan is to compare the sequence identified by Dr. Steve Wilton (Molecular Therapy, 2007) for skipping exon 50, H50A(+02+30), 5'-CCA CTC AGA GCT CAG ATC TTC TAA CTT CC-3' with that identified by Dr. Qi Lu (who will disclose his sequence to AVI soon). The strength of this approach is that independent evaluation utilizing different endpoints has been employed.

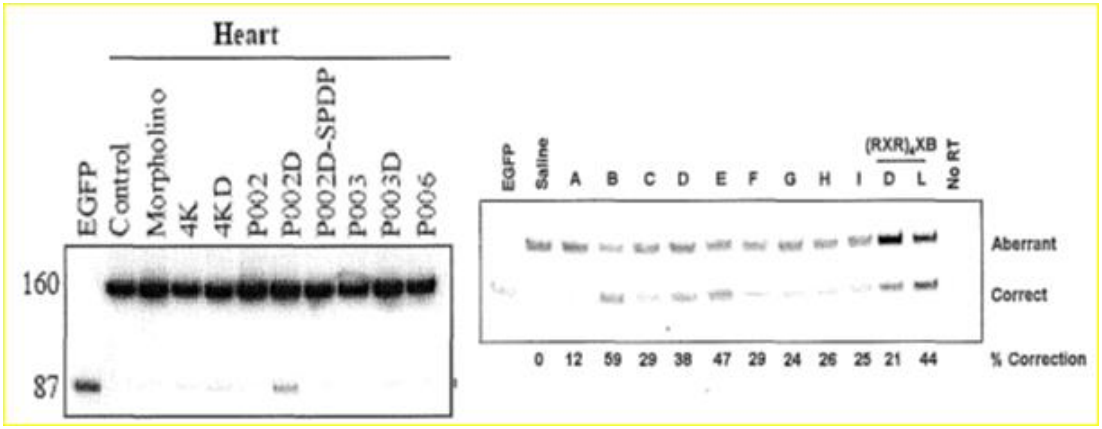
AVI BioPharma research and development group has been building a database of favorable PMO motifs for the past 10 years. This database has provided insights into novel rules for PMO self-interaction which will tend to exclude some selected sequences. We are currently updating this database so we can include evaluation of the selected sequences provided by Drs. Lu and Wilton.

B. Objective: Reduce dose through improved cell delivery and cell penetrance of PMOs.

AVI BioPharma has been developing peptide conjugates of PMOs for over 10 years in what has been referred to as the CytoPorter™ program. This strategy employs small peptides capable of enhanced cell penetration which are conjugated to the PMOs, referred to as PPMO. The program has utilized a "splice-correction" assay developed by Dr. Ryszard Kole which utilizes a cell line with a beta thalassemia cryptic intron splice acceptor and luciferase as a marker gene expression. This assay led to identification of the peptide $(RXR)_4XB$- in which R is arginine, X is aminohexanoic acid and B is beta alanine. This peptide conjugated to an antisense PMO directed to c-myc is currently the subject of a clinical study of coronary artery bypass grafts (CAGB). In that study, human saphenous veins are exposed to the study drug *ex vivo* just prior to engraftment into the coronary artery. This peptide has been evaluated for potential toxicity at AVI (Amantana, manuscript in press) and in GLP safety pharmacology studies. Further, Dr. Wilton has evaluated this peptide and found enhanced *in vivo* potency compared to unconjugated PMO. The limitations have been toxicity reported from animal studies at doses above 15mg/kg dose with repeated administration. However, it is likely that effective human doses will be significantly below this threshold. An evaluation of the published literature for the mdx mouse treated with PMOs suggests that a dose of 2 mg/kg may be therapeutic (see Section E). Since the PPMO compound has been shown to have greater potency than the corresponding PMO, it is reasonable to believe than an efficacious dose will be significantly lower than the maximum tolerated dose.

A comprehensive peptide structure activity study should involve *in vivo* efficacy and simple endpoint analysis. Collaboration with Dr. Kole over the past 2 years has utilized transgenic mice containing a gene with an artificial intron disruption of GFP expression. These studies have identified peptides which appear to be superior to (RXR)4XB-. The most notable to date has been (RXRRBR)2XB- which demonstrated greater splice correction activity in the heart yes it significantly less toxic than (RXR)4XB-.

This peptide has been provided to Drs. Wilton, Kole and Lu for evaluation in the mdx mouse. So far, Dr. Lu has reported a single dose of 30mg/kg of the PPMO is effective in the mdx mouse and no signs of toxicity have been observed. Dr. Lu indicates this is the smallest effective dose he has observed to date, which tends to confirm our estimations of the utility of PPMO. These studies will continue to evaluate a range of doses and repeated injections.



These figures come from Dr. Kole and show splice correction (lower band in each lane) in the heart of the EGFP mouse. On the left, only the P002 peptide synthesized with d-amino acids provided any splice correction in the heart. On the right, peptides B, D, E and (RXR)4XB all show significant splice correction in the heart. The peptide with the least toxicity and greatest efficacy from these effective peptides is peptide B which is (RXRRBR)2XB.

C. Objective: Identify modifications of PMOs that will enhance their inherent potency.

The highlights of the PMO platform technology are based on neutral charge and no metabolic degradation. Studies are in progress investigating the addition of a positively charged linkage in the PMO at selected sites, referred to as PMO+. This strategy has been used in treating non-human primates in Ebola lethal challenge studies, which have resulted in 70% dose reduction and improved long term survival over neutral PMO treatment. The observed affinity for a PMO binding to RNA is approximately 10^{-12} M and the PMO+ binding to RNA is approximately $10^{\bullet 13}$ M, which suggests the enhanced potency is due to greater binding affinity. We intend to evaluate the relative merit of this PMO+ modification for exon 50. The exon 23 sequences for the mdx mouse have been synthesized as PMO+ and are currently in purification at AVI. They will be sent shortly to Drs. Kole, Lu and Wilton for *in vivo* evaluation.

Finally, studies to date have shown synergy between the PPMO and PMO+ in what we refer to as PPMO+. These two modifications have the potential to reduce the systemic dose to 1% of the dose required for PMO compounds. The current optimal PPMO+ has been synthesized and is currently in purification. These mdx reagents will be sent to Drs. Kole, Lu and Wilton within the next few weeks for their evaluation in the mdx mouse.

D. Objective: Reduce dose through enhanced tissue-specific delivery.

We have initiated a collaboration with Dr. Hai Fang Yin in the laboratory of Dr. Matthew J.A. Wood at the University of Oxford. They recently reported use of a homing peptide conjugated to peptide nucleic acid agents. They have identified a muscle-specific homing peptide composed of NH2-ASSLNIA-COOH and more recently a 12-amino acid peptide with heart muscle specific tropism. These peptides may be useful in getting higher concentrations of PMOs into the most relevant tissues for treatment of DMD.

E. Objective: Estimate the dose and dose regimen based on pharmacokinetic studies.

A study conducted in rat involved IV and IM administration of a single 1.0, 5.0, 25.0, and 125.0 mg/kg of an exon 51 30-mer. The apparent plasma elimination half-life is non-linear but increases with increasing dose. The area under the plasma concentration versus time curve is also non-linear but would appear to reach a theoretical maximum of 43 µg-hr/ml. The volume of distribution shows a linear increase with dose exceeding 1 liter/kg at near a 10mg/kg dose, indicating more robust tissue accumulation at larger doses. The plasma pharmacokinetic data are shown in Table 1 below:

TABLE 1. Plasma Pharmacokinetics

Dose (mg/kg)	Route	Apparent Half-life (hr)	AUC (µg-hr/ml)	Vd (I/kg)	Clpl (ml/min)
1.0	IV	9.31	1.3	0.23	0.07
5.0	IV	22.78	14.38	0.73	0.17
25.0	IV	31.51	23.71	5.22	0.40
125.0	IV	39.84	39.31	27.88	2.20
1.0	IM	9.41	1.45	0.32	0.07
5.0	IM	7.31	11.79	3.33	1.01
25.0	IM	11.94	22.38	13.64	2.61
125.0	IM	15.85	38.49	75.79	10.89

We observed between 10 and 16% of the dose in the urine over the 24 hour post dose interval for IV administration. There is less than 0.2% of the dose excreted in feces so we estimate 84 to 90% of the administered dose in body tissues (Table 2).

TABLE 2. Renal Excretion Data

Dose (mg/kg)	Route	Urine Vol (ml-24hr)	Urine (mg)	% Dose Urine	Tissue (mg)	% Dose Tissue
1.0	IV	6.67	0.05	16.03	0.15	83.97
5.0	IV	22.46	0.44	12.30	0.56	87.70
25.0	IV	19.80	0.76	16.34	4.24	83.66
125.0	IV	6.80	2.65	10.00	22.35	90.00
1.0	IM	8.50	0.13	33.68	0.07	66.32
5.0	IM	15.33	0.14	10.21	0.86	89.79
25.0	IM	19.01	0.92	18.81	4.08	81.19
125.0	IM	16.40	8.38	3.81	16.62	96.19

The muscle accumulation was observed to be linear with dose for either IV or IM administration (r2= 0.999). A rat composed of 47.5% skeletal muscle (Even et al., 2001). Thus, a 250-gram rat has 119 grams (250g * 0.475 = 118.8g) of skeletal muscle. The pharmacokinetic studies utilized a 200mg (0.2g) sample of skeletal muscle recovered 24 hours after a single injection of H51A from either IV or IM routes of administration, homogenized in 1ml of PELB buffer and concentration determined by HPLC FDNA assay. Several of the muscle samples were below the detection limit of the HPLC assay so a liver to muscle ratio was calculated and applied to the lower doses to estimate muscle concentrations. The resulting muscle concentration in ng/ml is calculated [ng ecovery*0.2g/ml]/[0.475*250g]. The molar concentration is estimated from the molecular weight of the exon 51 compound studied (10,391.8 g/mol). We observed the compound in skeletal muscle as indicated in TABLE 3.

TABLE 3. Skeletal Muscle Recovery 24 hours Post Single Dose HSIA

Dose (mg/kg)	Route	Muscle (mg)	Muscle (ug/g)	Muscle µM	Muscle nM
1.0	IV	0.07	0.60	0.06	5.77
5.0	IV	0.27	2.24	0.22	21.55
25.0	IV	2.01	16.94	1.63	163.02
125.0	IV	10.61	89.39	8.60	860.16
1.0	IM	0.03	0.28	0.03	2.72
5.0	IM	0.41	3.46	0.33	33.29
25.0	IM	1.94	16.33	1.57	157.18
125.0	IM	7.89	66.48	6.40	639.70

There are no simple in vitro assay measures at present that can provide the EC50 for H51A, but based on our observations from cell culture (Wilton, et al, 2006) and other exon skipping inducing oligomers we believe this concentration will be near 100nM. The function of muscle may not require 100 percent exon skipping, so the 100nM value represents a feasible target tissue concentration. Based on the pharmacokinetic data, this would be observed following a dose of 15mg/kg. If we use the equivalent mg/m2 dose in a human (Freireich EJ., et al, 1966) we would administer 2.1mg/kg. In a 50kg boy this dose would be 105mg. This estimate is limited in that the pharmacokinetic studies were conducted in a healthy rat with normal muscle physiology. The muscle of the mdx mouse appears to be more permeable to the oligomer, which would suggest a smaller required dose to produce the 50 percent effect.

Studies reported in the peer reviewed literature utilized both intramuscular injections and systemic routes of administration to induce skipping of exon 23 in the mdx mouse. A summary of these studies is provided in Table 4 below:

TABLE 4. *In Vivo* Efficacy

Ref	Sequence	Chemistry	Dose	Route	Endpoints	Dose Eq.
Mann 2001	[+2-18]	2'0Me-LPF	1µg	IM	Western (WB)	143nM
Gebski 2003	[+7-18]	PMO-Leash	1µg	IM	WB-	116nM
Lu 2003 Nat	[+2-18]	2'0Me-BC	10µg	IM	WB, IHC, PCR	1.4µM
Wells 2003	[+2-18]	2'0Me-elect	8 µg	IM	WB, IHC	1.14 µM
Lu 2005	[+2-18]	2'0Me	100mg/kg	IV	IHC+	8.3mg/kg
Fletcher 2005	[+7-18]	PMO	25mg/kq	IP	IHC, WB, PCR	2.1mg/kg
Alter 2006	[+7-18]	PMO	100mg/kg	IV	IHC, WB, CK Force	8.3mg/kg
Fall 2006	[+2-18]	PMO	10µg	IM	IHC, WB, PCR	1.4µM

The estimate of the human dose equivalent in the right hand column is based on a 1-gram muscle diffusion area following the intramuscular (IM) injections and the molecular weight of the oligomer to arrive at a molar concentration. These observations indicate an efficacy range from 116 to 1400nM. The interpretation of these data is more complicated due to the use of lipofectins (LPF), block copolymers (BC), leashes and electroporation as delivery enhancers. However, the calculated concentration is based on 100 percent efficient delivery so these observations tend to

indicate efficacy with a PMO can be observed at concentrations as low as 116nM, which is in good agreement with efficacy in cell culture.

There are three studies which employed systemic delivery. The two intravenous (IV) studies employed 100mg/kg doses and the extravascular study used an intrperitoneal (IP) route with 25mg/kg. Robust efficacy was observed in each case and the anticipated human dose based on mg/m^2 dose scaling is 2.l mg/kg from an extravascular route of administration. This is in particularly good agreement with independent estimates based on pharmacokinetic observations.

The expression of functional Δ-dystrophin resulting from exon skipping will require active transcription of dystrophin and sufficient concentrations of exon-skipping oligomer in the nucleus of muscle cells. Once an oligomer has induced exon-skipping, the Δ-mRNA can be transported from the nucleus to the cytoplasm for translation. The steady-state of the functional Δ-dystrophin protein will be equal to the rate of translation of Δ-mRNA minus the rate of degradation of the Δ-dystrophin protein. The rate of transcription is relatively fast even for dystrophin, the largest transcript in the human genome, and is equal to 10 - 24 hours/copy. The rate of nuclear transport and the rate of translation are likely to be faster than the rate of transcription, on the order of 1 - 4 hours. Thus the critical features for accumulation of Δ-dystrophin protein will be the half-life of mRNA and the Δ-dystrophin protein.

The Δ-dystrophin protein in the mdx mouse following a single injection of exon skipping oligomer is about 12% of initial concentration at 12 weeks post dose. This would translate to approximately 3 half-lives and indicates a half-life of 4 weeks. (Lu et al., 2003). A single dose of PMO in the mdx mouse will create truncated mRNA that can be observed for over 4 weeks resulting in immunohistochemical evidence of Δ-dystrophin 8 weeks post dose (Fall et al., 2006). The time to steady-state expression of Δ-dystrophin protein will be equivalent to 3 to 5 half-lives of the protein, between 3 and 5 months. If dose is administered at protein half-life intervals, then the interval can be once per 4 weeks. This interval is further supported by the observation of Δ-mRNA persists for 4 weeks.

The apparent plasma half-life of the 30-mer targeting H51A shown in TABLE 1 is approximately 30 hours. The term "apparent" half-life indicates this is not likely to be the actual elimination half-life. Other pharmacokinetic studies with a variety of PMO sequences have plasma half-lives shorter than 30 hours but tissue residence times of 7 to 14 days. The anticipated muscle residence time of a PMO is expected to be greater than 7 days. This would mean a dose interval of 7 days would maintain muscle tissue levels and maximal muscle concentrations should be achieved in approximately one month.

Summary of the rational for the dose regimen:

1. The dose should be extravascular; eg. subcutaneous.

2. The dose should be 2.1 mg/kg for a 50 kg person or 105mg/dose.

3. The dose interval should be between 1 and 4 weeks.

III. Specific Aims of Future Studies (see attached Gantt chart for timelines)

Aim 1: Identify the optimal exon 50 sequence.

The ongoing efforts will provide direction for these studies. If Drs. Wilton and Lu select overlapping sequences, then AVI will prepare the selected sequence for confirmatory studies by both investigators with documented pure, high-quality controlled PMO.

If Wilton and Lu identify non-overlapping PMO sequences then a hierarchy of evaluation methods will be established at AVI to establish methods for comparison of the sequences that will utilize our database of favorable PMO traits to select optima from the unrelated sequences. Further, the potential for enhanced PMOs to alter the target optima can be addressed in cell culture, and Dr. Lu has offered to conduct these studies for exon 50.

Wilton: H50A(+02+30); 5'-CCACTCAGAGCTCAGATCTTCTAACTTCC-3'

Interpretation of data: If the sequences identified by the two independent investigators are identical, then no further interpretation is necessary. This singular sequence will be considered the lead exon 50 agent. If the sequences are not identical, then we will establish a small group of AVI investigators to evaluate the sequences with respect to unique experience and databases created at AVI. These will include:

a) Self-complementarity- this will have been screened by the investigators, but we have identified additional interactions unique to PMO chemistry. Sequences containing greater self- complementarity will receive a lower priority score.

b) Search for SNP- the sequences will be compared to snp databases to ensure there are no known polymorphisms in the targeted region.

c) *In vitro* translation database search for unfavorable sequence motifs- AVI BioPharma has developed a database of over 100 different sequences. These sequences differ by approximately 1000x in potency allowing us to evaluate unfavorable sequence motifs. Analysis of proposed sequences containing unfavorable motifs will receive lower priority scores.

d) Pharmacokinetic database- AVI BioPharma has developed a database of over 40 sequences that have been evaluated in pharmacokinetic studies. These sequences differ with respect to elimination half-life, renal clearance and volume of distribution, allowing the identification of both favorable and unfavorable motifs. Analysis of proposed sequences containing unfavorable motifs will receive lower priority scores.

e) BLAST search of GenBank for potential interactions with other human genes. Any 14 contiguous base match will receive a lower priority score.

In addition to technical criteria, intellectual property issues will be considered in selecting which sequence to prioritize.

Aim 2: Identify best PMO modality for exon skipping in the mdx mouse model.

The comparison studies for exon 23 in the mdx mouse model are in progress in three laboratories. The results should provide an estimate of the relative potency for PMO, PPMO, PMO+ and PPMO+. The emphasis of the three laboratories tend to differ, and we intend to leave experimental detail to each individual laboratory as a means of providing a greater range of experience. We anticipate the PPMO+ will be the most effective modality. If this is not clearly established, then the lead agent for exon 50 will be the modality with the lowest toxicity and for which we have the greatest experience. The methodology will be reviewed for the experiments to either identify areas of conflict or provide greater confidence due to confirmation.

Use mdx mouse as simple screening tool to compare potency of multiple versions of PMOs:

PMO	anticipate efficacy at 150mg/kg
PMO+	anticipate efficacy at 150mg/kg
PPMO	anticipate efficacy at 15mg/kg
PPMO+	anticipate efficacy at 3mg/kg

This set of studies is currently in progress as materials have been prepared and initial shipments made to Drs. Wilton, Lu, and Kole. Dr. Lu has conducted a pilot study with a single 30mg/kg dose of PPMO and reported by phone that he is seeing robust exon skipping 4 weeks after a single dose. He indicated this is more potent in the mdx mouse than any agents he has evaluated to date and is currently evaluating the uniformity of muscle response. Dr. Wilton has provided initial observations with the PMO+ which clearly show greater potency than the PMO in the mdx mouse. Dr. Kole will meet at AVI next week and discuss his observations with the molecular endpoint of splice altering.

Based on the results from work under Aims 1 and 2, AVI will select a lead compound to take forward into initial pharmacokinetic and toxicity testing described in Aim 4.

Aim 3: Evaluate the influence of homing peptides for PMOs

In parallel with evaluation of the lead compound, additional work will be conducted with another technology that could increase the potency and effectiveness of exon-skipping PMOs.
The muscle homing peptide (MHP), NH_2-ASSLNIA-COOH, was identified using a phage display method (Samoylova and Smith 1999). These studies involved intravenous administration of $>10^{12}$ phage virions (a phage display library) to mice and recovery of phage associated with skeletal muscle. The associated phage were amplified and a second round of selection conducted to identify the 7-amino acid MHP peptide. It is not clear that the small MHP will promote cell penetration, so we propose to combine the MHP with our cell penetrating peptides (CPP) in the following matrix of studies:

MHP-PM0-3'

5'-PMO-MHP

CPP-PMO-MHP

MHP-PMO-CPP

MHP-CPP-PM0-3'

CPP-MHP-PM0-3' 5'-

PMO-CPP-MHP

5'-PMO-MHP-CPP

A peptide that was identified using the same phage display technique with homing to the pancreatic vasculature, NH_2-CRV ASVLPC-COOH (Kolonin et al., 2006). This peptide binds to the prolactin receptor (PRLR). We intend to use this peptide as a control for tissue specificity for the MHP. Since the matrix of 8 possibilities in combination with CPP is possible, we will conduct an initial set of studies to evaluate cell uptake into cultured myotomes first. The PMO sequence will be designed to induce exon skipping of dystrophin and the success of the studies will be based on observation of PCR amplified, truncated dystrophin transcripts. Once favorable uptake and efficacy are observed, then the control will be prepared with the same orientations of PMO, CPP and homing peptide.

The optimal MHP/PRLR, CPP and PMO combinations will be evaluated in the mdx mouse targeting skipping of exon 23. If the dose required to induce functional dystrophin production is reduced by a factor of 3, then this approach will be considered sufficiently useful for further evaluation.

Collaborative studies with Dr. HaiFang Yin in the laboratory of Matthew Wood at the University of Oxford will utilize a 12-amino acid peptide they have identified with homing properties to the heart muscle. These studies will follow those proposed to evaluate the MHP. The purpose of these studies will be to investigate the potential therapeutic delivery of exon skipping PMOs to the heart. If successful, this strategy may be used to treat or prevent specific areas of deficit in exon skipping therapy for DMD.

Aim 4. Evaluate the pharmacokinetics and toxicity of selected lead compound

Initial animal studies with the lead compound selected in Aim 2 will utilize doses and dose regimens that are aggressive and based on successful historical animal model studies, e.g. in the range from 30 to 150mg/kg.

AVI BioPharma has substantial experience with studies in rats for initial evaluation of pharmacokinetics. Specifically we will:

1) Develop and validate analytical methodology that is sufficiently sensitive, specific and reproducible to allow measurement and quantitation of candidates agents in body fluids and tissues of animals. A platform method referred to as the FDNA assay has been developed for virtually any PMO agent and utilizes a fluorescently labeled DNA with sequence complementary to the candidate PMO. This method has also been utilized in the case of Ebola virus to simultaneously identify three PMO sequences administered to the animal at the same time. The method is generally

sensitive enough to detect 10ng/ml, is linear with less than 5% coefficient of variance and is reproducible.

2) Conduct plasma protein binding studies and stability studies in various biological fluids and tissues.

3) Determine solubility of these candidates at 30mg/ml or greater. Any candidate that is not soluble at 30mg/ml will be re-synthesized and re-evaluated. If the candidate is confirmed to be poorly soluble (the only cases of this have involved high guanine content), then synthesis of the candidate with inosine substitution of guanine one position at a ti will be conducted to improve solubility.

4) Determine plasma and urinary concentrations of candidates as various times after administration to rats by various routes including intravenous, subcutaneous and intraperitoneal. The data will be fit to pharmacokinetic models and relevant pharmacokinetic parameters (including half-life, renal clearance, volume of distribution, mean residence time and area under the curve) for each route of administration.

5) Tissue distribution studies will also be conducted at various times after administration of the candidate with particular attention to skeletal muscle and the heart muscle, and

6) No PMO metabolite has been detected to date, but this can only be meaningful if studies are sufficient for a mass balance study (e.g., account for greater than 75% of the injected candidate). The studies conducted in rats will also provide data for body and organ weights, blood chemistry, and histology, which will guide future understanding of the potential toxicology of each candidate. These studies will be particularly useful when new PMO modalities (PPMO, PMO+ and PPMO+) are evaluated in an effort to optimize the therapeutic candidate's efficacy.

Both Drs. Lu and Wilton are also interested in studying gene expression array analysis of PMOs used to induce skipping of exon 50 in a mouse model. This methodology relies on comparison of differences in gene expression between untreated and mice treated chronically with PMOs.

Aim 5: Combination sequences leading to skipping exons 50 - 53.

As is the case with Aim 3, the proposed work under this aim is to continue to research options for the best long-term therapeutic options for exon 50 DMD patients. It is not on the critical path to clinical testing of the initial exon 50 drug candidate, but is proposed to be conducted in parallel.

Dr. Wilton has identified a strategy to skip exons 50-53 and in the near term exons 50-51.
The goal of these studies will be to search for combinations of PMOs that work in synergy. Thus, the total dose required to skip multiple exons should be less than the sum of the doses required to skip them individually. Discussions with Dr. Wilton indicate he is currently supported to evaluate the "cocktail" approach, so our intention is to support his efforts with the most optimal PMO

modalities, experimental design support when needed, pharmacokinetic studies, and analytical support.

Aim 6: GMP Manufacture and GLP Safety Pharmacology

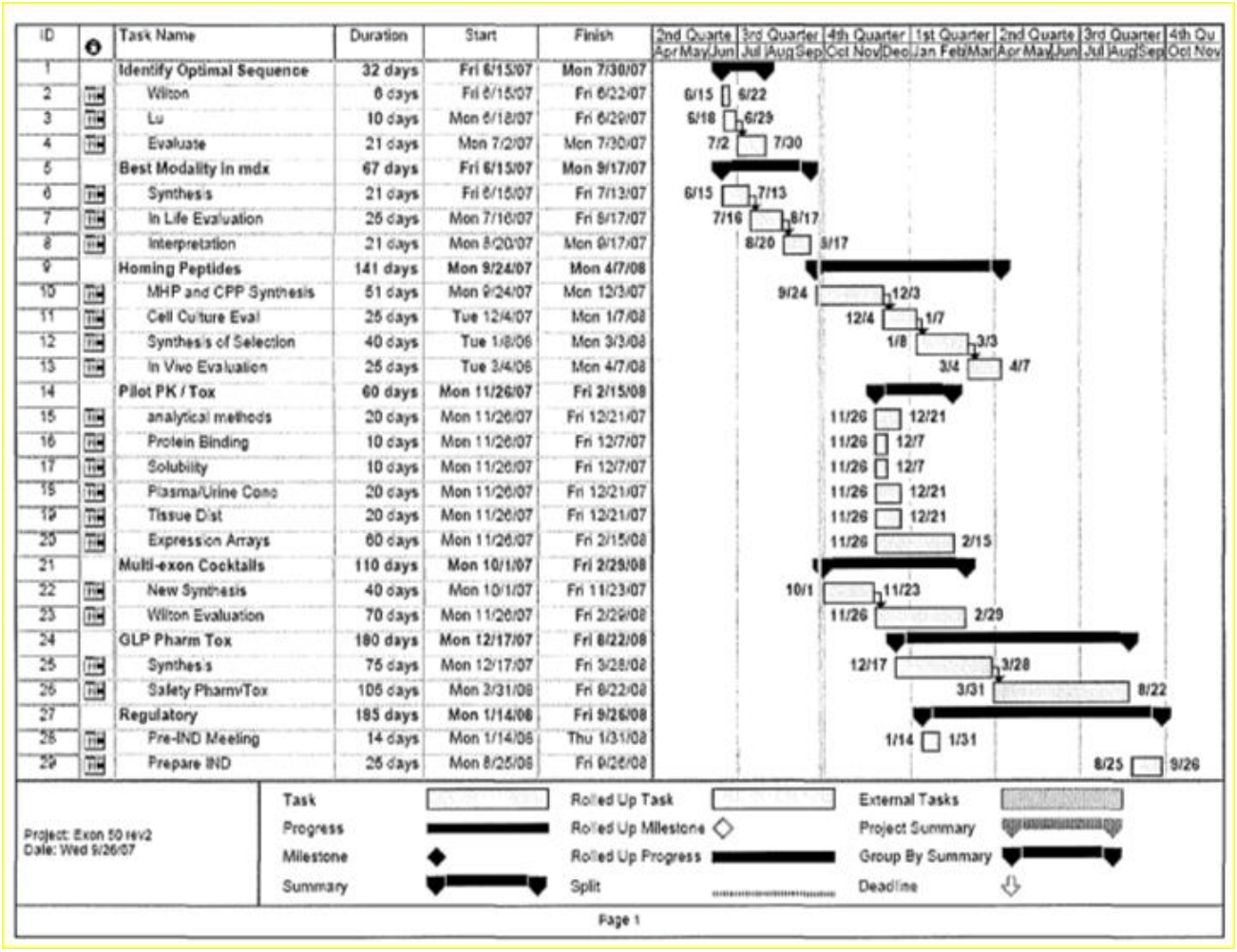
The goal here is to synthesize sufficient quantities of the lead compound for further characterization, including efficacy and formal GLP toxicity testing in *in vivo* models. While process development is generally advanced at AVI, we will need to evaluate QA/QC methods and methods for recovery, characterization, purification, stability assessment specifically for the selected compound.

The second goal is to perform required benchmarks for IND filing and moving the drug candidate into Phase I/II clinical trials. These studies involve conventional safety pharmacology studies, including a cardiovascular study (in monkeys), pulmonary function study (in monkeys), renal function study (in monkeys), central nervous system (in rats), pharmacokinetic studies in monkeys, conventional toxicology (two species will be required), and genotoxicity studies. Traditional genotoxicity, Ames assay and SCE and micronucleus assays in T-cells are not adequate to evaluate the genotoxicity of the relatively high molecular weight DMD agents. We know these agents do not enter bacterial cells of the Ames assay or T-cells utilized in the other assays. Hence, we propose use of zebrafish embryos for genotoxicity evaluation. This can be done at Oregon State University, a regional center of excellence in zebrafish studies.

Our experience with PMOs suggests a very safe profile and minimal requirement for safety pharmacology studies. However, those agents with newer modalities are likely to require a greater degree of safety pharmacology and toxicology investigation. The specific design of the safety pharmacology and GLP tox studies will be based on the modality chosen and the results obtained in the preliminary pharmacokinetic and toxicity studies conducted in Aim 4.

Aim 7: Prepare and File IND

Based on preclinical safety and efficacy results, AVI's clinical and regulatory departments will prepare an IND to support initial human clinical testing of the selected exon 50 drug candidate.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1 Identify Optimal Sequence 32 days Fri 6/15/07 Mon 7/30/07
2 Wilton 6 days Fri 6/15/07 Fri 6/22/07
3 Lu 10 days Mon 6/18/07 Fri 6/29/07
4 Evaluate 21 days Mon 7/2/07 Mon 7/30/07
5 Best Modality in mdx 67 days Fri 6/15/07 Mon 9/17/07
6 Synthesis 21 days Fri 6/15/07 Fri 7/13/07
7 In Life Evaluation 25 days Mon 7/16/07 Fri 8/17/07
8 Interpretation 21 days Mon 8/20/07 Mon 9/17/07
9 Homing Peptides 141 days Mon 9/24/07 Mon 4/7/08
10 MHP and CPP Synthesis 51 days Mon 9/24/07 Mon 12/3/07
11 Cell Culture Eval 25 days Tue 12/4/07 Mon 1/7/08
12 Synthesis of Selection 40 days Tue 1/8/08 Mon 3/3/08
13 In Vivo Evaluation 25 days Tue 3/4/08 Mon 4/7/08
14 Pilot PK / Tox 60 days Mon 11/26/07 Fri 2/15/08
15 analytical methods 20 days Mon 11/26/07 Fri 12/21/07
16 Protein Binding 10 days Mon 11/26/07 Fri 12/7/07
17 Solubility 10 days Mon 11/26/07 Fri 12/7/07
18 Plasma/Urine Conc 20 days Mon 11/26/07 Fri 12/21/07
19 Tissue Dist 20 days Mon 11/26/07 Fri 12/21/07
20 Expression Arrays 60 days Mon 11/26/07 Fri 2/15/08
21 Multi-exon Cocktails 110 days Mon 10/1/07 Fri 2/29/08
22 New Synthesis 40 days Mon 10/1/07 Fri 11/23/07
23 Wilton Evaluation 70 days Mon 11/26/07 Fri 2/29/08
24 GLP Pharm Tox 180 days Mon 12/17/07 Fri 8/22/08
25 Synthesis 75 days Mon 12/17/07 Fri 3/28/08
26 Safety Pharm/Tox 105 days Mon 3/31/08 Fri 8/22/08
27 Regulatory 185 days Mon 1/14/08 Fri 9/26/08
28 Pre-IND Meeting 14 days Mon 1/14/08 Thu 1/31/08
29 Prepare IND 25 days Mon 8/25/08 Fri 9/26/08
6/15 6/22
6/18 6/29
7/2 7/30
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7/16 8/17
8/20 9/17
9/24 12/3
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1/14 1/31
8/25 9/26
Project: Exon 50 rev2
Date: Wed 9/26/07
Task
Progress
Milestone
Summary
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Rolled Up Milestone
Rolled Up Progress
Split
External Tasks
Project Summary
Group By Summary
Deadline
Page 1

APPENDIX B

Project Cost Estimates

Aim 1: Identify the optimal exon 50 sequence

Materials:	**$5,000**
Labor:	**$5,000**
Total:	**$10,000**

Aim 2: Identify best PMO modality for exon skipping in the mdx mouse model.

Materials:	(already synthesized)
Labor:	$2,500
Total:	$2,500

Aim 3: Evaluate the influence of homing peptides for PMOs

Materials:	$15,000
Animal studies:	$5,000
Labor:	$2,500
	$22,500

Aim 4: Evaluate the pharmacokinetics and toxicity of optimal PMO modalities (PPMO, PMO+ and PPMO+)

Materials:	$28,000
Animals:	$1,000
Labor:	$30,000
Total:	$59,000

Aim 5: Combination sequences leading to skipping exons 50-53

Materials:	$20,000
Labor:	$5,000
Total	$25,000

Aim 6: GMP Manufacture and GLP Safety Pharmacology

Materials:	$1,100,000
GLP tox studies	$1,150,000
Genotox study:	$30,000
Labor:	$20,000
Total:	$2,300,000

Aim 7: Prepare and File IND

Labor:	$33,000

Total Estimated Cost, All Aims:	**$2,452,000**

FIRST AMENDMENT TO SPONSORED RESEARCH AGREEMENT

This FIRST AMENDMENT TO SPONSORED RESEARCH AGREEMENT (the "Amendment") is entered into effective as of May 28, 2009 ("Amendment Date"), by and between AVI BioPharma, Inc., an Oregon corporation having offices at 4575 SW Research Way, Suite 200, Corvallis, OR 97333 (the "Company"), and Charley's Fund, Inc., a 501(c)(3) tax-exempt public non-profit organization with a mailing address of P.O. Box 297, South Egremont, MA 01258 (the "Sponsor") (each a "Party" and together the "Parties"), and amends that certain SPONSORED RESEARCH AGREEMENT, effective as of October 12, 2007, by and between the Parties (the "Agreement"), as follows.

RECITALS

WHEREAS, the Parties acknowledge that additional funding is necessary to complete the Research Project (as that term is defined in the Agreement), and therefore desire to amend the Agreement to increase the amount of the Project Funds (as that term is defined in the Agreement) to be provided to the Company by the Sponsor;

WHEREAS, the Parties desire to revise certain terms under the Agreement as they relate to payments to be made by the Company to the Sponsor, and

WHEREAS, the Parties desire to revise and update the description of the Research Project and the milestones contemplated therein,

NOW, THEREFORE, for good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, the Parties hereto agree as follows:

1. Section 1.11 is amended and restated in its entirety as follows:

"Net Sales" means the gross amount invoiced for sales of Research Products by (i) for purposes of Section 9 hereof, Sponsor, its Affiliates, and sublicensees or (ii) for purposes of Section 4.3.1 hereof, Company, its Affiliates and licensees, in any case to an independent third party in an arms-length transaction, less:

(a) Trade, quantity and cash discounts allowed;

(b) Discounts, refunds, rebates, chargebacks, retroactive price adjustments, and any other allowances which effectively reduce the net selling price;

(c) Credits for actual Research Product returns; and

(d) Any tax imposed on the production, sale, delivery or use of the Research Product, including, without limitation, sales, use, excise, or value added taxes.

2. Section 4.1 is amended and restated in its entirety as follows:

Subject to the terms and conditions of this Agreement including the repayment rights provided for in Section 4.3, the Sponsor shall pay the Company a total amount of Five Million Dollars ($5,000,000.00) which amount is inclusive of all direct costs of Research Project activities (the "Project Funds") as follows:

(a) The parties acknowledge and agree that Two Million Dollars ($2,000,000.00) of the Project Funds have been paid to the Company as of May 26, 2009 of which approximately One Million Three Hundred Fifty Thousand Dollars ($1,350,000.00) have been spent and earned by the Company hereunder. The parties further acknowledge and agree that, as of such date, the Company has completed each of Aim 1, Aim 2, Aim 3 and Aim 5 as set forth in the Study Protocol. The remaining Six Hundred Fifty Thousand Dollars ($650,000.00) of unspent, but received Project Funds shall be allocated as follows: (a) approximately Three Hundred Thousand Dollars ($300,000.00) shall be spent connection with the NHP multi dose confirmation study (as further described in the Study Protocol), and (b) approximately Three Hundred Fifty Thousand

Dollars ($350,000.00) shall be spent in connection with cGMP manufacture of drug product, AVI-5038 (as further described in the Study Protocol), and, in both cases, such funds shall be recognized as earned upon the initiation of these studies.

(b) The Sponsor shall pay the remaining Three Million Dollars ($3,000,000.00) of the Project Funds to the Company in accordance with the following schedule of events (each, as further described in the Study Protocol):

(i) One Hundred Thousand Dollars ($100,000.00) upon initiation of GLO genotoxicity study;

(ii) Six Hundred Fifty Thousand Dollars ($650,000.00) upon initiation of the OLP toxicology study in mdx mice;

(iii) One Million Six Hundred Twenty-five Thousand Dollars ($1,625,000.00) upon initiation of the GLP NHP toxicology study;

(iv) Three Hundred Thousand Dollars ($300,000.00) upon initiation of the GLP safety pharmacology study; and

(v) Three Hundred Twenty-five Thousand Dollars ($325,000.00) upon submission of an IND.

Besides the direct costs for the above studies, allowable costs include the costs for the stability testing, and fill/finish of drug product, which are taken into account in the above figures.

The Sponsor shall not be obligated to make any payments to the Company in addition to those set forth in this Section 4.1 unless the parties otherwise mutually agree in writing.

3. Section 4.3.1 is amended and restated in its entirety as follows:

A total royalty of 5% of Net Sales shall be paid to the Sponsor by the Company, less any portion of the Project Funds already repaid to the Sponsor by the Company. In no event shall royalties payable to the Sponsor exceed the total amount of Project Funds actually provided by the Sponsor to the Company. Such royalty shall be payable on a calendar quarter basis, within 45 days after the end of each quarter.

4. Section 4.3.2 is deleted.

5. Section 4.3.3 is deleted.

6. Section 4.3.5 is amended and restated in its entirety as follows:

Without limiting the foregoing, in the event that the full amount of the Project Funds have not been repaid to the Sponsor at first commercial sale into a Major Market of the Research Product via the payment mechanisms of Section 4.3.4, the Company shall make payments to the Sponsor as provided for in Section 4.3.1.

7. Each of Appendix A and Appendix B is amended and restated in its entirety as set forth on Schedule I and Schedule II, respectively, attached to the Amendment.

8. The Parties acknowledge and agree that Dr. Steven Shrewsbury, CMO, is currently serving as the Principal Investigator.

9. All capitalized terms not defined herein shall have the meanings ascribed to them in the Agreement. This Amendment is hereby incorporated into the Agreement. Except as specifically modified herein, the Agreement remains in full force and effect without further modification.

IN WITNESS WHEREOF, the Parties hereto have entered into this Amendment as of the date first written above.

AVI BIOPHARMA, INC.

By: /s/ Leslie Hudson
Name: Leslie Hudson
Title: CEO President

CHARLEY'S FUND, INC.

By: /s/ Benjamin D. Seckler
Name: Benjamin D. Seckler
Title: President

SCHEDULE I

Study Protocol

In addition to being subject to change as may be agreed upon by the parties, the Study Protocol described below may be modified by AVI as is scientifically appropriate and otherwise reasonable based on results obtained or additional information that becomes available during its execution.

GLP Toxicology Plan

12-month toxicology study in mdx mice

- *mdx* mice do not express dystrophin, eliminating "on-target" issues
- *mdx* mice have pathology, but vehicle arm should be sufficient control
  - Increased CK, muscle pathology etc.
- Regimen: To achieve adequate total exposure
- Dose range: 1.5 log range (top dose ~48 mg/kg)
- 3-Month take out to enable IND

Group	Animals/ Group (M/F)	Study Drug				Necropsy (M/F) (days)			
		Name	Dose (mg/kg)	Route	Freq.	79 12 doses +1 day	106 12 doses +28 days	359 52 doses +1 day	386 52 doses +28 days
1	24/0	AVI-5038	0	IV	TBD, 360 days	6/0	6/0	6/0	6/0
2	24/0		Low	IV		6/0	6/0	6/0	6/0
3	24/0		Medium	IV		6/0	6/0	6/0	6/0
4	24/0		Interm.	IV		6/0	6/0	6/0	6/0
5	24/0		High	IV		6/0	6/0	6/0	6/0

12-month toxicology study in healthy cynomolgus monkey

- Regimen: To achieve adequate total exposure
- Dose range: 1 log range (top dose ~20 mg/kg)
- 3-Month take out to enable IND

Group	Animals/ Group (M/F)	Study Drug				Necropsy (M/F) (days)			
		Name	Dose (mg/kg)	Route	Freq.	79 12 doses +1 day	106 12 doses +28 days	359 52 doses +1 day	386 52 doses +28 days
1	12/0	AVI-5038	0	IV	TBD, 360 days	3/0	3/0	3/0	3/0
2	12/0		Low	IV		3/0	3/0	3/0	3/0
3	12/0		Medium	IV		3/0	3/0	3/0	3/0
4	12/0		High	IV		3/0	3/0	3/0	3/0

Genotoxicity

- Ames Assay
- Chromosomal Aberration Assay
- Mouse Micronucleus Test

Safety Pharmacology

- Cynomolgus monkey and mouse
- 3 i.v. doses - Low, medium, high

Prepare and File IND

Based on preclinical safety and efficacy results, an IND will be prepared to support initial human clinical testing of the exon 50 drug candidate. It is anticipated that this will occur in or about May, 2010.

SCHEDULE II

Because the parties acknowledge that sufficient financial detail is provided directly in the text of the First Amendment to the Agreement, this schedule has been intentionally left blank.

Exhibit 10.4

SEVENTH AMENDMENT TO LEASE

THIS SEVENTH AMENDMENT TO LEASE (this "**Seventh Amendment**") is made as of April 27, 2018, by and between **ARE-MA REGION NO. 38, LLC**, a Delaware limited liability company ("**Landlord**"), and **SAREPTA THERAPEUTICS, INC.**, a Delaware corporation ("**Tenant**").

RECITALS

A. Landlord and Tenant are parties to that certain Lease Agreement dated as of June 25, 2013, as amended by that certain First Amendment to Lease dated as of November 13, 2013 ("**First Amendment**"), as further amended by that certain Second Amendment to Lease dated as of February 18, 2014, as further amended by that certain Third Amendment to Lease dated as of July 31, 2014 ("**Third Amendment**"), as further amended by that certain Fourth Amendment to Lease dated as of August 28, 2014, and as further amended by that certain Fifth Amendment to Lease dated as of November 7, 2014, and as further amended by that certain Sixth Amendment to Lease dated as of November 30, 2016 (the "**Sixth Amendment**") (as amended, the "**Lease**"). Pursuant to the Lease, Tenant leases certain premises containing approximately 88,459 rentable square feet (the "**Existing Premises**") in a building located at 215 First Street, Cambridge, Massachusetts. The Existing Premises are more particularly described in the Lease. Capitalized terms used herein without definition shall have the meanings defined for such terms in the Lease.

B. Landlord and Tenant desire, subject to the terms and conditions set forth below, to amend the Lease to, among other things, expand the size of the Existing Premises by adding approximately 63,698 rentable square feet of office space in the Building.

NOW, THEREFORE, in consideration of the foregoing Recitals, which are incorporated herein by this reference, the mutual promises and conditions contained herein, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, Landlord and Tenant hereby agree as follows:

1. **Sixth and Seventh Expansion Premises**. In addition to the Existing Premises, (a) commencing on the Sixth Expansion Premises Commencement Date (as defined below), Landlord leases to Tenant, and Tenant leases from Landlord, the "**Sixth Expansion Premises**" consisting of (i) that certain portion of the fourth floor of the Building consisting of approximately 28,258 rentable square feet (the "**Fourth Floor Sixth Expansion Premises**"), (ii) that portion of the lower level of the Building consisting of approximately 2,395 rentable square feet (the "**Lower Level Sixth Expansion Premises**"), (iii) that portion of the third floor of the Building consisting of approximately 29,352 rentable square feet (the "**Third Floor Sixth Expansion Premises**"), and (iv) that portion of the first floor of the Building consisting of approximately 883 rentable square feet (the "**First Floor Sixth Expansion Premises**"), and (b) commencing on the Seventh Expansion Premises Commencement Date (as defined below), Landlord leases to Tenant, and Tenant leases from Landlord, the "**Seventh Expansion Premises**" consisting of that certain portion of the first floor of the Building, consisting of approximately 2,810 rentable square feet, all as shown on **Exhibit A** attached hereto.

2. **Delivery**.

a. **Sixth Expansion Premises**. The "**Sixth Expansion Premises Commencement Date**" shall be the day that is 1 business day after the mutual execution and delivery of this Seventh Amendment by the parties. Landlord shall deliver the Sixth Expansion Premises to Tenant on the Sixth Expansion Premises Commencement Date free of all tenants and occupants, broom clean and free of debris and personal property. The "**Third Floor/First Floor Sixth Expansion Premises Rent Commencement Date**" shall be July 1, 2018. Tenant shall commence paying Base Rent with respect to the Third Floor Sixth Expansion Premises and the First Floor Sixth Expansion Premises on the Third Floor/First Floor Sixth Expansion Premises Commencement Date. The "**Fourth Floor/Lower Level Sixth Expansion Premises Rent Commencement Date**" shall be

October 1, 2018. Tenant shall commence paying Base Rent with respect to the Fourth Floor Sixth Expansion Premises and the Lower Level Sixth Expansion Premises on the Fourth Floor/Lower Level Sixth Expansion Premises Rent Commencement Date.

Except as set forth in this Seventh Amendment: (i) Tenant shall accept the Sixth Expansion Premises in their "as-is" condition as of the Sixth Expansion Premises Commencement Date; (ii) Landlord shall have no obligation for any defects in the Sixth Expansion Premises (but the foregoing language shall not relieve Landlord from any or its maintenance obligations under the Lease); and (iii) Tenant's taking possession of the Sixth Expansion Premises shall be conclusive evidence that Tenant accepts the Sixth Expansion Premises and that the Sixth Expansion Premises were in good condition at the time possession was taken.

b. **Seventh Expansion Premises**. Landlord shall use reasonable efforts to deliver the Seventh Expansion Premises to Tenant free of all tenants and occupants, broom clean and free of debris and personal property on or before November 1, 2018. If Landlord fails to timely deliver the Seventh Expansion Premises, Landlord shall not be liable to Tenant for any loss or damage resulting therefrom, and the Lease with respect to the Seventh Expansion Premises shall not be void or voidable. Notwithstanding anything to the contrary contained herein, if Landlord fails to Deliver the Seventh Expansion Premises to Tenant by the December 1, 2018 (as such date may be extended for Force Majeure delays, the "**Abatement Date**"), then, unless such failure is due to a delay caused by Tenant, Base Rent payable with respect to the Seventh Expansion Premises commencing on the Seventh Expansion Premises Rent Commencement Date (as defined below) shall be abated 1 day for each day after the Abatement Date (as such date may be amended for Force Majeure delays) that Landlord fails to Deliver the Seventh Expansion Premises to Tenant in the condition required above.

The "**Seventh Expansion Premises Commencement Date**" shall be the day that Landlord delivers the Seventh Expansion Premises to Tenant free of all tenants and occupants, broom clean and free of debris and personal property. The "**Seventh Expansion Premises Rent Commencement Date**" shall be the date that is 3 months after the Seventh Expansion Premises Commencement Date.

For the period of 60 consecutive days after the Seventh Expansion Premises Commencement Date, Landlord shall, at its sole cost and expense (which shall not constitute an Operating Expense), be responsible for any repairs that are required to be made to the Building Systems serving the Seventh Expansion Premises, unless Tenant or any Tenant Party was responsible for the cause of such repair, in which case Tenant shall pay the cost.

Except as set forth in this Seventh Amendment: (i) Tenant shall accept the Seventh Expansion Premises in their "as-is" condition as of the Seventh Expansion Premises Commencement Date; (ii) Landlord shall have no obligation for any defects in the Seventh Expansion Premises (but the foregoing language shall not relieve Landlord from any or its maintenance obligations under the Lease); and (iii) Tenant's taking possession of the Seventh Expansion Premises shall be conclusive evidence that Tenant accepts the Seventh Expansion Premises and that the Seventh Expansion Premises were in good condition at the time possession was taken.

c. **General**. Upon the request of Landlord, Tenant shall execute and deliver a written acknowledgment of the Sixth Expansion Premises Commencement Date, the Third Floor/First Floor Sixth Expansion Premises Rent Commencement Date, the Fourth Floor/Lower Level Sixth Expansion Premises Rent Commencement Date, the Seventh Expansion Premises Commencement Date and the expiration date of the Lease in substantially the form of the "Acknowledgement of Commencement Date" attached to the Lease as **Exhibit D**; provided,

however, Tenant's failure to execute and deliver such acknowledgment shall not affect Landlord's rights hereunder.

Tenant agrees and acknowledges that, except as otherwise set forth in this Seventh Amendment, neither Landlord nor any agent of Landlord has made any representation or warranty with respect to the condition of all or any portion of the Sixth Expansion Premises or the Seventh Expansion Premises and/or the suitability of the Sixth Expansion Premises or the Seventh Expansion Premises for the conduct of Tenant's business, and Tenant waives any implied warranty that the Sixth Expansion Premises or the Seventh Expansion Premises are suitable for the Permitted Use.

3. **Premises**.

a. Commencing on the Sixth Expansion Premises Commencement Date, the defined terms for "**Premises**" and "**Rentable Area of Premises**" on page 1 of the Lease are deleted in their entirety and replaced with the following:

"**Premises:** That portion of the Project containing approximately 149,347 rentable square feet, consisting of (i) approximately 32,314 rentable square feet of laboratory and office space on the first and second floors of the Building, (ii) approximately 14,062 rentable square feet of office space on the fourth floor of the Building, (iii) approximately 15,077 rentable square feet of office space located on the fourth floor of the Building ("**Expansion Premises**"), (iv) approximately 2,038 rentable square feet located on the lower level of the Building ("**Second Expansion Premises A**"), (v) approximately 1,993 rentable square feet located on the lower level of the Building ("**Second Expansion Premises B**"), (vi) approximately 4,445 of laboratory and office space on the lower level of the Building ("**Third Expansion Premises**"), (vii) approximately 7,461 rentable square feet of space on the first and second floors of the Building (the "**Fourth Expansion Premises**"), (viii) approximately 11,069 rentable square feet of space on the first floor of the Building (the "**Fifth Expansion Premises**"), and (ix) the "**Sixth Expansion Premises**" consisting of (A) approximately 28,258 rentable square feet on the fourth floor of the Building (the "**Fourth Floor Sixth Expansion Premises**"), (B) approximately 2,395 rentable square feet on the lower level of the Building ("**Lower Level Sixth Expansion Premises**"), (C) approximately 29,352 rentable square feet on the third floor of the Building (the "**Third Floor Sixth Expansion Premises**"), and (D) approximately 883 rentable square feet on the first floor of the Building (the "**First Floor Sixth Expansion Premises**"), all as shown on **Exhibit A**. The portions of the Premises reflected in sections (i) and (ii) above shall be collectively referred to herein as the '**Original Premises**' and the portions of the Premises reflected in Sections (iv) and (v) shall be collectively referred to herein as the '**Second Expansion Premises**'."

"**Rentable Area of Premises:** 149,347 sq. ft."

As of the Sixth Expansion Premises Commencement Date, **Exhibit A** to the Lease shall be amended to include the Sixth Expansion Premises described on **Exhibit A** attached to this Seventh Amendment.

b. Commencing on the Seventh Expansion Premises Commencement Date, the defined terms for "**Premises**" and "**Rentable Area of Premises**" on page 1 of the Lease are deleted in their entirety and replaced with the following:

"**Premises:** That portion of the Project containing approximately 152,157 rentable square feet, consisting of (i) approximately 32,314 rentable square feet of laboratory and office space on the first and second floors of the Building, (ii) approximately 14,062 rentable square feet of office space on the fourth floor of the Building, (iii) approximately 15,077 rentable square feet of office space located on the fourth floor of the Building ("**Expansion**

Premises"), (iv) approximately 2,038 rentable square feet located on the lower level of the Building ("**Second Expansion Premises A**"), (v) approximately 1,993 rentable square feet located on the lower level of the Building ("**Second Expansion Premises B**"), (vi) approximately 4,445 of laboratory and office space on the lower level of the Building ("**Third Expansion Premises**"), (vii) approximately 7,461 rentable square feet of space on the first and second floors of the Building (the "**Fourth Expansion Premises**"), (viii) approximately 11,069 rentable square feet of space on the first floor of the Building (the "**Fifth Expansion Premises**"), (ix) the "**Sixth Expansion Premises**" consisting of (A) approximately 28,258 rentable square feet on the fourth floor of the Building (the "**Fourth Floor Sixth Expansion Premises**"), (B) approximately 2,395 rentable square feet on the lower level of the Building ("**Lower Level Sixth Expansion Premises**"), (C) approximately 29,352 rentable square feet on the third floor of the Building (the "**Third Floor Sixth Expansion Premises**"), and (D) approximately 883 rentable square feet on the first floor of the Building (the "**First Floor Sixth Expansion Premises**"), and (x) the "**Seventh Expansion Premises**" consisting of approximately 2,810 rentable square feet, all as shown on **Exhibit A**. The portions of the Premises reflected in sections (i) and (ii) above shall be collectively referred to herein as the '**Original Premises**' and the portions of the Premises reflected in Sections (iv) and (v) shall be collectively referred to herein as the '**Second Expansion Premises**'."

"**Rentable Area of Premises:** 152,157 sq. ft."

As of the Seventh Expansion Premises Commencement Date, **Exhibit A** to the Lease shall be amended to include the Seventh Expansion Premises described on **Exhibit A** attached to this Seventh Amendment.

4. **Base Rent**.

a. **Existing Premises**. Tenant shall continue to pay Base Rent with respect to the Existing Premises as provided in the Lease through January 31, 2021. On February 1, 2021, Tenant shall pay Base Rent with respect to the Existing Premises as provided in the schedule of Base Rent attached hereto as **Exhibit E**. On each February 1st (each, a "**Seventh Amendment Adjustment Date**") occurring thereafter, (i) Base Rent payable with respect to the Original Premises, the Fourth Expansion Premises and the Fifth Expansion Premises shall be increased by multiplying the Base Rent payable with respect to the Original Premises, the Fourth Expansion Premises and the Fifth Expansion Premises immediately before such Seventh Amendment Adjustment Date by 2% and adding the resulting amount to the Base Rent payable with respect to the Original Premises, the Fourth Expansion Premises and the Fifth Expansion Premises immediately before such Seventh Amendment Adjustment Date, and (ii) Base Rent payable with respect to the Second Expansion Premises and the Third Expansion Premises shall be increased by multiplying the Base Rent payable with respect to the Second Expansion Premises and the Third Expansion Premises immediately before such Seventh Amendment Adjustment Date by 3% and adding the resulting amount to the Base Rent payable with respect to the Second Expansion Premises and the Third Expansion Premises immediately before such Seventh Amendment Adjustment Date.

b. **Third Floor/First Floor Expansion Premises**. Beginning on the Third Floor/First Floor Sixth Expansion Premises Rent Commencement Date, Tenant shall pay Base Rent with respect to the Third Floor Sixth Expansion Premises and the First Floor Sixth Expansion Premises in the amount of $53.00 per rentable square foot of the Third Floor Sixth Expansion Premises and the First Floor Sixth Expansion Premises per year. Base Rent payable with respect to the Third Floor Sixth Expansion Premises and the First Floor Sixth Expansion Premises shall be increased on each Seventh Amendment Adjustment Date following the Third Floor/First Floor Sixth Expansion Premises Rent Commencement Date by 2% and adding the resulting amount to the Base Rent payable with respect to the Third Floor Sixth Expansion Premises and the First Floor Sixth Expansion Premises immediately before such Seventh Amendment Adjustment Date.

c. **Fourth Floor/Lower Level Sixth Expansion Premises**. Beginning on the Fourth Floor/Lower Level Sixth Expansion Premises Rent Commencement Date, Tenant shall pay Base Rent with respect to the Fourth Floor Sixth Expansion Premises in the amount of $43.00 per rentable square foot of the Fourth Floor Sixth Expansion Premises per year. Base Rent payable with respect to the Fourth Floor Sixth Expansion Premises shall be increased on each Seventh Amendment Adjustment Date following the Fourth Floor/Lower Level Sixth Expansion Premises Rent Commencement Date by 2% and adding the resulting amount to the Base Rent payable with respect to the Fourth Floor Sixth Expansion Premises.

Beginning on the Fourth Floor/Lower Level Sixth Expansion Premises Rent Commencement Date, Tenant shall pay Base Rent with respect to the Lower Level Sixth Expansion Premises in the amount of $30.00 per rentable square foot of the Lower Level Sixth Expansion Premises per year. Base Rent payable with respect to the Lower Level Sixth Expansion Premises shall be increased on each Seventh Amendment Adjustment Date following the Sixth Expansion Premises Rent Commencement Date by 3% and adding the resulting amount to the Base Rent payable with respect to the Lower Level Sixth Expansion Premises.

d. **Seventh Expansion Premises**. Beginning on the Seventh Expansion Premises Rent Commencement Date, Tenant shall pay Base Rent with respect to the Seventh Expansion Premises in the amount of $54.06 per rentable square foot of the Seventh Expansion Premises per year. Base Rent payable with respect to the Seventh Expansion Premises shall be increased on each Seventh Amendment Adjustment Date following the Seventh Expansion Premises Commencement Date by 2% and adding the resulting amount to the Base Rent payable with respect to the Seventh Expansion Premises immediately before such Seventh Amendment Adjustment Date.

e. **Base Rent Schedule**. A schedule of Base Rent is attached hereto as **Exhibit E**.

5. **Tenant's Share**.

a. Commencing on the Third Floor/First Floor Sixth Expansion Premises Rent Commencement Date, the defined term "**Tenant's Share of Operating Expenses**" on page 1 of the Lease is deleted in its entirety and replaced with the following:

"**Tenant's Share of Operating Expenses:** 32.37%"

b. Commencing on the Fourth Floor/Lower Level Sixth Expansion Premises Rent Commencement Date, the defined term "**Tenant's Share of Operating Expenses**" on page 1 of the Lease is deleted in its entirety and replaced with the following:

"**Tenant's Share of Operating Expenses:** 40.72%"

c. Commencing on the Seventh Expansion Premises Commencement Date, the defined term "**Tenant's Share of Operating Expenses**" on page 1 of the Lease is deleted in its entirety and replaced with the following:

"**Tenant's Share of Operating Expenses:** 41.49%"

6. **Term**.

a. **Base Term**. The Base Term of the Lease is hereby extended through the "**Expiration Date**," which shall be September 30, 2025.

b. **Right to Extend Term**. For the avoidance of doubt, Section 40 of the original Lease shall continue in full force and effect provided, however, that Tenant may only exercise its Extension Rights (i) for the first Extension Term, with respect to not less than 75% of the entire then-existing Premises being leased by Tenant on the date that Tenant delivers written notice of its election to exercise its first Extension Right (provided that the Premises with respect to which the Lease is not extended shall, in Landlord's reasonable discretion, be marketable to third parties taking into account all relevant factors such as size, configuration and location of such Premises), and (ii) for the second Extension Term, with respect to not less than 100% of the entire then-existing Premises being leased by Tenant on the date that Tenant delivers written notice of its election to exercise its second Extension Right. The definition of "**Market Rate**" set forth in Section 40(a) is hereby deleted in its entirety and replaced with the following: "**Market Rate**" shall mean the rate that comparable landlords of comparable buildings have accepted in current transactions from non-equity (i.e., not being offered equity in the buildings) and nonaffiliated tenants of similar financial strength for space of comparable size, quality (including all Tenant Improvements, Alterations and other improvements) in comparable Class A lab/office buildings in East Cambridge for a comparable term, with the determination of the Market Rate to take into account all relevant factors, including Tenant's then-current use of each different portion of the Premises as laboratory or office space, tenant inducements, parking costs, proximity to amenities and public transit, leasing commissions, allowances or concessions, if any.

7. **Security Deposit**.

a. Commencing on the date of this Seventh Amendment, the defined term "**Security Deposit**" on Page 1 of the Lease is deleted in its entirely and replaced with the following:

"**Security Deposit:** $1,000,000"

Landlord currently holds a Security Deposit of $646,974 under the Lease. Concurrently with Tenant's delivery of a signed original of this Seventh Amendment to Landlord, Tenant shall deliver to Landlord an amended Letter of Credit which increases the amount of the existing Letter of Credit being held by Landlord to $1,000,000 or an additional Letter of Credit in the amount of $353,026.

b. If, as of July 1, 2020, (i) Tenant is not in Default of the Lease, (ii) Tenant has not been in Default of this Lease at any time during the previous 12 months of the Term, and (iii) Tenant's can reasonably demonstrate to Landlord that Tenant's net worth as of July 1, 2020, is substantially similar to Tenant's net worth as of the date of this Seventh Amendment (collectively, the "**Reduction Requirements**" and each a "**Reduction Requirement**"), then the Security Deposit shall be reduced to $500,000 (the "**Reduced Security Deposit**"). If Tenant provides Landlord with a written request to Landlord for such reduction of the Security Deposit and is not then in Default of the Lease, then, so long as all of the Reduction Requirements have been met, Landlord shall cooperate with Tenant, at no cost, expense or liability to Landlord, to reduce the Letter of Credit then held by Landlord to the amount of the Reduced Security Deposit. If the Security Deposit is reduced as provided herein, then from and after the date of such reduction, the "**Security Deposit** " shall be deemed to be the Reduced Security Deposit, for all purposes of this Lease.

8. **Seventh Amendment Tenant Improvements**. Tenant shall have the right to construct certain tenant improvements in the Premises subject to the terms of the "**Work Letter**" attached hereto as **Exhibit C**.

Tenant shall not be required to remove or restore the Seventh Amendment Tenant Improvements (as defined in the Work Letter) at the expiration or earlier termination of the Term, nor shall Tenant have the right to remove any of the Seventh Amendment Tenant Improvements at any time during the Term or at the expiration or earlier termination of the Term.

9. **Staircase Allowance Rent**. In addition to the Tenant Improvement Allowance (as defined in the Work Letter), Landlord shall, subject to the terms of the Work Letter, make available to Tenant the Staircase Allowance (as defined in the Work Letter). Commencing on the first day of the month following the date that Landlord first disburses all or any portion of the Staircase Allowance, and continuing thereafter on the first day of each month of the Term, Tenant shall pay the amount necessary to amortize the portion of the Staircase Allowance actually funded by Landlord, if any, over a period of 10 years in equal monthly payments with interest at a rate of 7% per annum, which interest shall begin to accrue on the date that Landlord first disburses the applicable portion of such Staircase Allowance. Tenant acknowledges that because the Staircase Allowance may be disbursed to Tenant in multiple disbursements following the date of this Seventh Amendment, the Additional Rent payable by Tenant pursuant to this Section 9 may be adjusted following each such disbursement. Any of the Staircase Allowance and applicable interest remaining unpaid as of the expiration or earlier termination of the Lease shall be paid to Landlord in a lump sum at the expiration or earlier termination of this Lease; provided, however, if Tenant exercises its first Extension Right pursuant to Section 40(a) of the original Lease then, commencing on the first day of the Extension Term, Tenant shall have no further obligation to reimburse Landlord for the Staircase Allowance or applicable interest remaining.

10. **Maintenance**. Notwithstanding the foregoing, Tenant has the right to elect to undertake, at Tenant's sole cost and expense, all of Landlord's maintenance obligations with respect to the Building Systems serving exclusively the Premises (the "**Exclusive Systems**") in the condition which the Exclusive Systems are required to be maintained by Landlord under the Lease. If Tenant elects to undertake Landlord's maintenance obligations with respect to the Exclusive Systems, Tenant shall continue to perform all of Landlord's maintenance obligations with respect to the Exclusive Systems until the date that Landlord undertakes the maintenance of the Exclusive Systems following a Maintenance Breach. The maintenance obligations described in the first sentence of this paragraph shall include, without limitation, an obligation on the part of Tenant to repair, replace and maintain the Exclusive Systems in good condition and working order and in a first class manner consistent with other Class A office and laboratory projects in the Cambridge area. Tenant's maintenance obligation shall also include the procurement and maintenance of contracts, in form and substance reasonably satisfactory to Landlord, with copies to Landlord upon Landlord's written request, for and with contractors acceptable to Landlord specializing and experienced in the maintenance and repair that Tenant is responsible for under the Lease. During any period where Tenant is maintaining the Exclusive Systems as provided for in this paragraph, Landlord shall, notwithstanding anything to the contrary contained in the Lease, have no obligation to perform any maintenance, repairs or replacements under the Lease with respect to the Exclusive Systems. Tenant's maintenance obligations under this paragraph shall not include the right on the part of Tenant to make any capital repairs or improvements to the Exclusive Systems without Landlord's prior written consent. Tenant shall not take or omit to take any action, the taking or omission of which shall cause waste, damage or injury to the Project or any other tenants of the Project. If Tenant (a) fails to maintain any portion of the Exclusive Systems in a manner reasonably acceptable to Landlord within the requirements of the Lease, (b) Tenant's maintenance performed pursuant to this paragraph adversely affects the Building, Building Systems other than the Exclusive Systems, or other tenants of the Project, or (c) if Robert Fay ceases to be employed by Tenant (each, a "**Maintenance Breach**"), Landlord shall have the right to provide Tenant with written notice thereof and to assume maintenance of all or any portion of the Exclusive Systems if Tenant does not cure the Maintenance Breach within 10 business days after receipt of such notice.

11. **Parking**.

a. Commencing on the Third Floor/First Floor Sixth Expansion Premises Rent Commencement Date, subject to the terms of Section 7(b) of the Sixth Amendment, Landlord shall make available to Tenant at then-current market rates (which market rate may be adjusted from time to time) a license for up to an additional 28 parking spaces in the Binney Parking Garage located at 50-60 Binney Street ("**Third Floor/First Floor Parking Spaces**"), all of such parking

spaces to be on a non-reserved basis. Tenant shall notify Landlord prior to the Third Floor/First Floor Sixth Expansion Premises Rent Commencement Date as to how many of the Third Floor/First Floor Parking Spaces Tenant will license pursuant to the Lease. As of the Third Floor/First Floor Sixth Expansion Premises Rent Commencement Date, the market parking rate for the parking spaces is $300.00 per parking space month. Tenant's pro rata share of the Binney Parking Garage shall be adjusted in accordance with the number of Third Floor/First Floor Parking Spaces licensed by Tenant during the Term pursuant to this Section 11(a).

b. Commencing on the Fourth Floor/Lower Level Sixth Expansion Premises Rent Commencement Date, subject to the terms of Section 7(b) of the Sixth Amendment, Landlord shall make available to Tenant at then-current market rates (which market rate may be adjusted from time to time) a license for up to an additional 27 parking spaces in the Binney Parking Garage located at 50-60 Binney Street ("**Fourth Floor/Lower Level Parking Spaces**"), all of such parking spaces to be on a non-reserved basis. Tenant shall notify Landlord prior to the Fourth Floor/Lower Level Sixth Expansion Premises Rent Commencement Date as to how many of the Fourth Floor/Lower Level Parking Spaces Tenant will license pursuant to the Lease. As of the Fourth Floor/Lower Level Sixth Expansion Premises Rent Commencement Date, the market parking rate for the parking spaces is $300.00 per parking space month. Tenant's pro rata share of the Binney Parking Garage shall be adjusted in accordance with the number of Fourth Floor/Lower Level Parking Spaces licensed by Tenant during the Term pursuant to this Section 11(b).

c. In the event that the Premises are increased to include any of the Identified Space (as defined in Section 14 below), upon the commencement date of the Lease with respect to such Identified Space, Tenant's pro rata share of the Binney Parking Garage shall be further adjusted by an amount equal to 0.9 parking spaces per rentable square foot of the applicable Identified Space. Also, if Tenant surrenders any portion of the Premises at any time during the Term, Tenant's pro share of the Binney Parking Garage shall be decreased by an amount equal to 0.9 parking spaces per rentable square foot of the surrendered portion of the Premises.

12. **Intentionally Omitted**.

13. **Signage**. Tenant shall have the right, subject to Landlord's signage program at the Project and all applicable Legal Requirements, to remove Tenant's existing second story Exterior Sign from its current location on the First Street side of the Building and to replace such sign with a new Exterior Sign at the leftmost corner of the First Street side of the Building at the highest location allowable permitted in the zoning district in which the Premises are located. Tenant's Exterior Sign shall continue to be subject to the terms of Section 10 of the First Amendment (as the same has been amended).

Landlord shall, at Landlord cost and expense, provide signage on the Building directories with respect to the Sixth Expansion Premises and the Seventh Expansion Premises. Tenant shall continue to have Building Entrance Signage pursuant to Section 10 of the First Amendment. So long as Tenant remains the largest tenant at the Project in terms of rentable square footage leased, Tenant shall have the most prominent listing in all Building directories.

14. **Right to Expand**.

a. **Expansion in the Building**. Subject to the superior rights of any tenants of the Building existing as of the date of this Seventh Amendment, Tenant shall have the right, but not the obligation, during the Term, to expand the Premises (the "**Expansion Right**") to include any Available Space in the Building upon the terms and conditions in this Section. For purposes of this Section 14(a), "**Available Space**" shall mean that certain space located on the second and third floors of the Building described on **Exhibit D** attached hereto, which is not occupied by an existing tenant or which is occupied by a tenant and such then tenant does not wish to renew (whether or

not such tenant has a right to renew) its occupancy of such space. If there is any Available Space, Landlord shall, at such time as Landlord shall elect so long as Tenant's rights hereunder are preserved, deliver to Tenant written notice (the "**Expansion Notice**") of such Available Space ("**Identified Space**"), together with the terms and conditions (including base rent) on which Landlord is prepared to lease Tenant such Identified Space, provided that the base rent payable with respect to the Identified Space shall be at the Market Rate (as defined in Section 6(b) above. Tenant shall be entitled to exercise its right under this Section 14(a) only with respect to the entire Identified Space described in such Expansion Notice. Tenant shall have 10 days following delivery of the Expansion Notice to deliver to Landlord written notification of Tenant's exercise of the Expansion Right with respect to the Identified Space ("**Exercise Notice**"). If the parties are unable to agree on the Market Rate payable with respect to the Identified Space within 30 days after Tenant's delivery to Landlord of an Exercise Notice, then the Market Rate payable with respect to Identified Space shall be determined by arbitration pursuant to Section 40(b) of the original Lease. The term of the Lease with respect to the Available Space shall be co-terminous with the Term of the Lease for the existing Premises; provided, however, that (1) if less than 3 years are remaining in the Base Term of the Lease at the time Landlord delivers an Expansion Notice, then Tenant's exercise of the Expansion Right with respect to the Identified Space shall be contingent on Tenant's exercising its first Extension Right simultaneously with its Exercise Notice, and (2) if less than 3 years are remaining in the first Extension Term at the time Landlord delivers an Expansion Notice, then Tenant's exercise of the Expansion Right with respect to the Identified Space shall be contingent on Tenant's exercising its second Extension Right simultaneously with its Exercise Notice. Tenant's failure to deliver an Exercise Notice to Landlord shall be deemed to be an election by Tenant not to exercise Tenant's Expansion Right with respect to the Identified Space, in which case Landlord shall have the right to lease the Identified Space to any third party on any terms and conditions acceptable to Landlord; provided, however, that if (x) Landlord intends to lease the Identified Space to a third party for less than ninety percent (90%) of the net effective rent contained in the Expansion Notice, or (y) Landlord fails to enter into an agreement to lease the Identified Space within 9 months after Landlord's delivery of the applicable Expansion Notice to Tenant, then prior to leasing the Identified Space to a third party, Landlord shall again give Tenant an Expansion Notice and Tenant shall again have its Expansion Right with respect to such Identified Space, subject to the terms and conditions of this Section 14(a). Notwithstanding anything to the contrary contained herein, Tenant's Expansion Right shall be null and void and of no further force or effect after (i) the date that is 9 months prior to the expiration date of the Base Term if Tenant has not exercised its first Extension Right pursuant to Section 40(a) of the original Lease, and (ii) the date that is 9 months prior to the expiration of the first Extension Term if Tenant has not exercised its second Extension Right pursuant to Section 40(a) of the original Lease.

b. **Amended Lease**. If: (i) Tenant fails to timely deliver notice accepting the terms of an Expansion Notice, or (ii) after the expiration of a period of 15 days after Landlord's delivery to Tenant of a proposed amendment to this Lease, no lease amendment for the Identified Space acceptable to both parties each in their reasonable discretion has been executed, Tenant shall be deemed to have forever waived its right to lease such Identified Space; provided, however, that so long as the parties are diligently negotiating the terms of such proposed amendment in good faith, such 15 day period shall be extended on a day-for-day basis until either an amendment is executed by the parties or the date either party elects to cease negotiating an amendment.

c. **Exceptions**. Notwithstanding the above, the Expansion Right shall, at Landlord's option, not be in effect and may not be exercised by Tenant:

(i) during any period of time that Tenant is in Default under any provision of the Lease; or

(ii) if Tenant has been in Default under any provision of the Lease 3 or more times, whether or not the Defaults are cured, during the 12 month period prior to the date on which Tenant seeks to exercise the Expansion Right.

d. **Termination**. The Expansion Right shall, at Landlord's option, terminate and be of no further force or effect even after Tenant's due and timely exercise of the Expansion Right if, after such exercise, but prior to the commencement date of the lease of such Available Space, (i) Tenant fails to timely cure any default by Tenant under the Lease; or (ii) Tenant has Defaulted 3 or more times during the period from the date of the exercise of the Expansion Right to the date of the commencement of the lease of the Available Space, whether or not such Defaults are cured.

e. **Subordinate**. Tenant's rights in connection with the Expansion Right are and shall be subject to and subordinate to any existing expansion or extension rights granted to any existing tenant of the Building.

f. **Rights Personal**. The Expansion Right is personal to Tenant and are not assignable without Landlord's consent, which may be granted or withheld in Landlord's sole discretion separate and apart from any consent by Landlord to an assignment of Tenant's interest in the Lease, except that they may be assigned in connection with any Permitted Assignment of this Lease.

g. **No Extensions**. The period of time within which the Expansion Right may be exercised shall not be extended or enlarged by reason of Tenant's inability to exercise the Expansion Right.

15. **Security**. Upon written request from Tenant, Landlord shall cause, at Tenant's sole cost and expense, the Building elevators to be set to provide card access to floors of the Building which are leased entirely to Tenant. In addition, Tenant may, at Tenant's sole cost and expense, shall have the right to utilize the First Floor Sixth Expansion Premises as a branded (which branding shall be subject to Landlord's approval, which approval shall not be unreasonably withheld, conditioned or delayed), dedicated security checkpoint for Tenant's visitors and employees. Any alterations or improvements required in order to convert the First Floor Sixth Expansion Premises to a checkpoint shall constitute Alterations subject to Section 12 of the original Lease, including Landlord's right to approve Tenant's plans for the conversion.

16. **Lobby Improvements**. Landlord shall, at Landlord's sole cost and expense, make improvements to the main lobby area of the Building, as determined by Landlord in its sole and absolute discretion, during the calendar year 2018. Landlord shall provide Tenant with a copy of Landlord's plans for such lobby improvements once they have been finalized by Landlord.

17. **Brokers**. Landlord and Tenant each represents and warrants that it has not dealt with any broker, agent or other person (collectively, "**Broker**") in connection with the transaction reflected in this Seventh Amendment and that no Broker brought about this transaction, other than CBRE New England. Landlord and Tenant each hereby agrees to indemnify and hold the other harmless from and against any claims by any Broker, other than CBRE New England, claiming a commission or other form of compensation by virtue of having dealt with Tenant or Landlord, as applicable, with regard to this leasing transaction.

18. **Miscellaneous**.

a. This Seventh Amendment is the entire agreement between the parties with respect to the subject matter hereof and supersedes all prior and contemporaneous oral and written agreements and discussions. This Seventh Amendment may be amended only by an agreement in writing, signed by the parties hereto.

b. This Seventh Amendment is binding upon and shall inure to the benefit of the parties hereto and their respective successors and assigns.

c. This Seventh Amendment may be executed in any number of counterparts, each of which shall be deemed an original, but all of which when taken together shall constitute one and the same

instrument. The signature page of any counterpart may be detached therefrom without impairing the legal effect of the signature(s) thereon provided such signature page is attached to any other counterpart identical thereto except having additional signature pages executed by other parties to this Seventh Amendment attached thereto.

d. Except as amended and/or modified by this Seventh Amendment, the Lease is hereby ratified and confirmed and all other terms of the Lease shall remain in full force and effect, unaltered and unchanged by this Seventh Amendment. In the event of any conflict between the provisions of this Seventh Amendment and the provisions of the Lease, the provisions of this Seventh Amendment shall prevail. Whether or not specifically amended by this Seventh Amendment, all of the terms and provisions of the Lease are hereby amended to the extent necessary to give effect to the purpose and intent of this Seventh Amendment.

[Signatures are on the next page.]

IN WITNESS WHEREOF, the parties hereto have executed this Seventh Amendment as of the day and year first above written.

TENANT:

SAREPTA THERAPEUTICS, INC.,
a Delaware corporation

/s/ Douglas S. Ingram
By: Douglas S. Ingram
Its: President and CEO

LANDLORD:

ARE-MA REGION NO. 38, LLC,
a Delaware limited liability company

By: Alexandria Real Estate Equities, L.P.,
a Delaware limited partnership, managing member

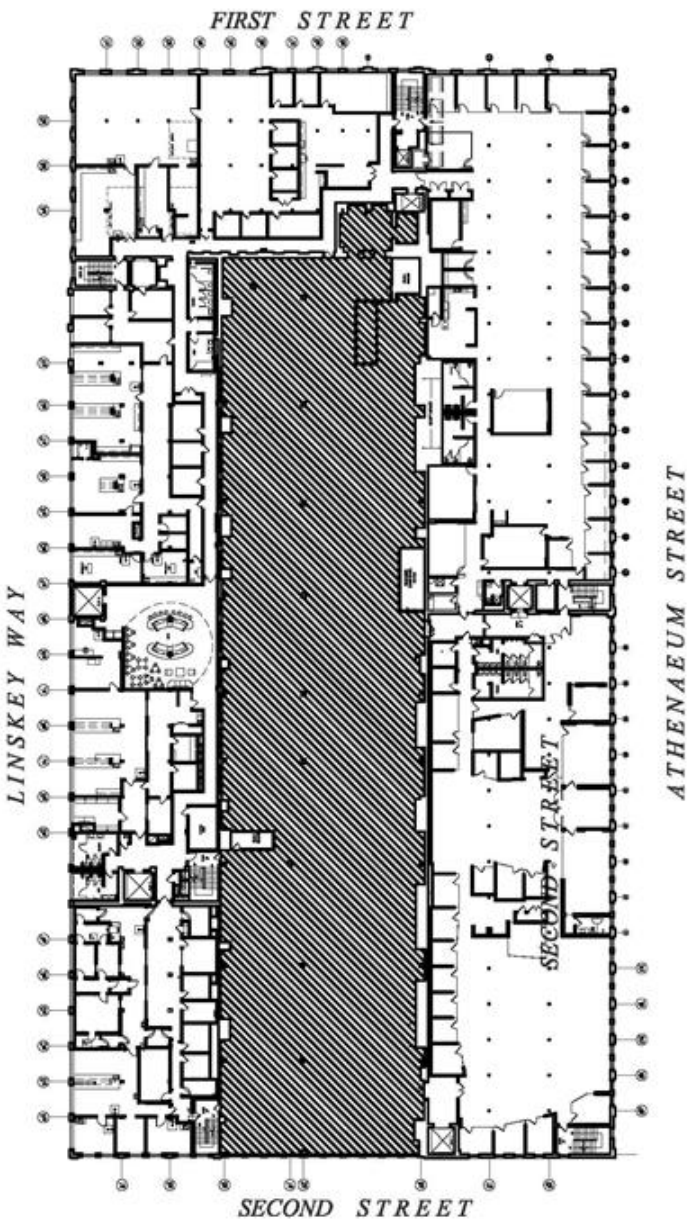
By: ARE-QRS Corp.,
a Maryland corporation, general partner

/s/ Jackie Clem
By: Jackie Clem
Its: Senior VP RE Legal Affairs

Exhibit A

Sixth Expansion Premises and Seventh Expansion Premises

215 FIRST STREET
CAMBRIDGE, MASSACHUSETTS

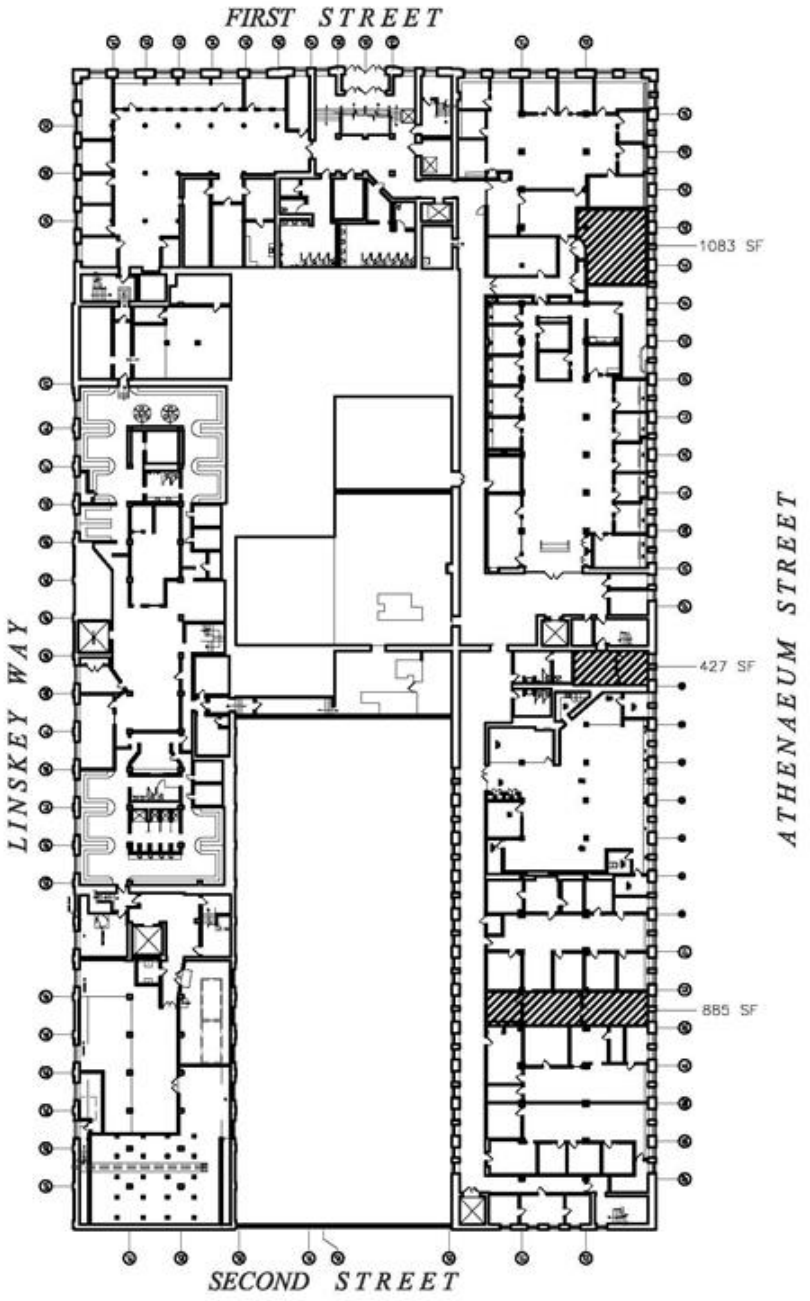


FLOOR 4

= FOURTH FLOOR SIXTH EXPANSION PREMISES – 28,258 RSF

SAREPTA THERAPEUTICS

215 FIRST STREET
CAMBRIDGE, MASSACHUSETTS

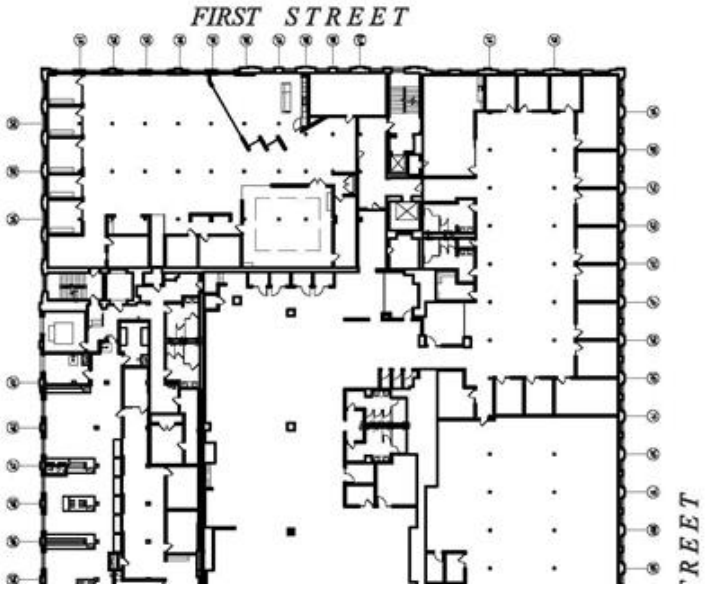


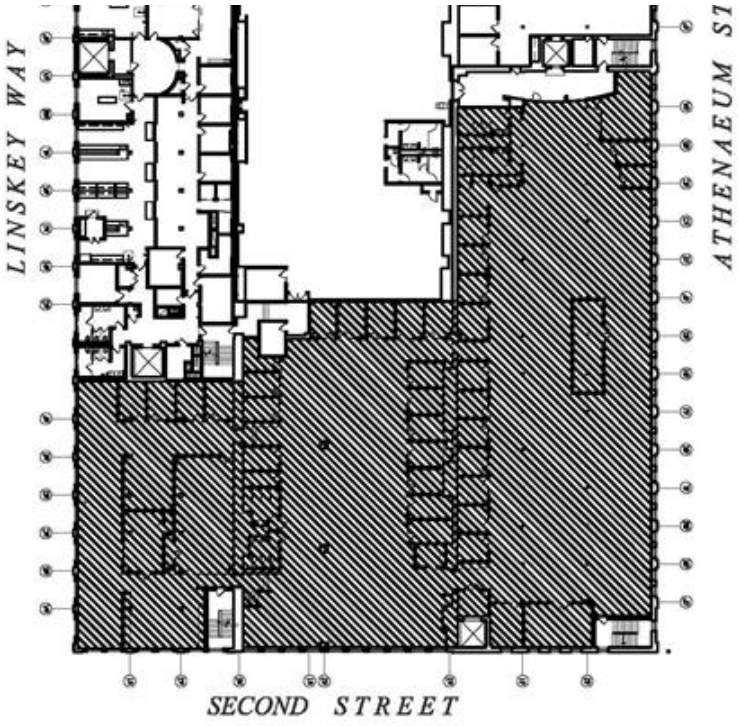
LOWER LEVEL

– LOWER LEVEL SIXTH EXPANSION PREMISES – 2,395 RSF

SAREPTA THERAPEUTICS

215 FIRST STREET
CAMBRIDGE, MASSACHUSETTS



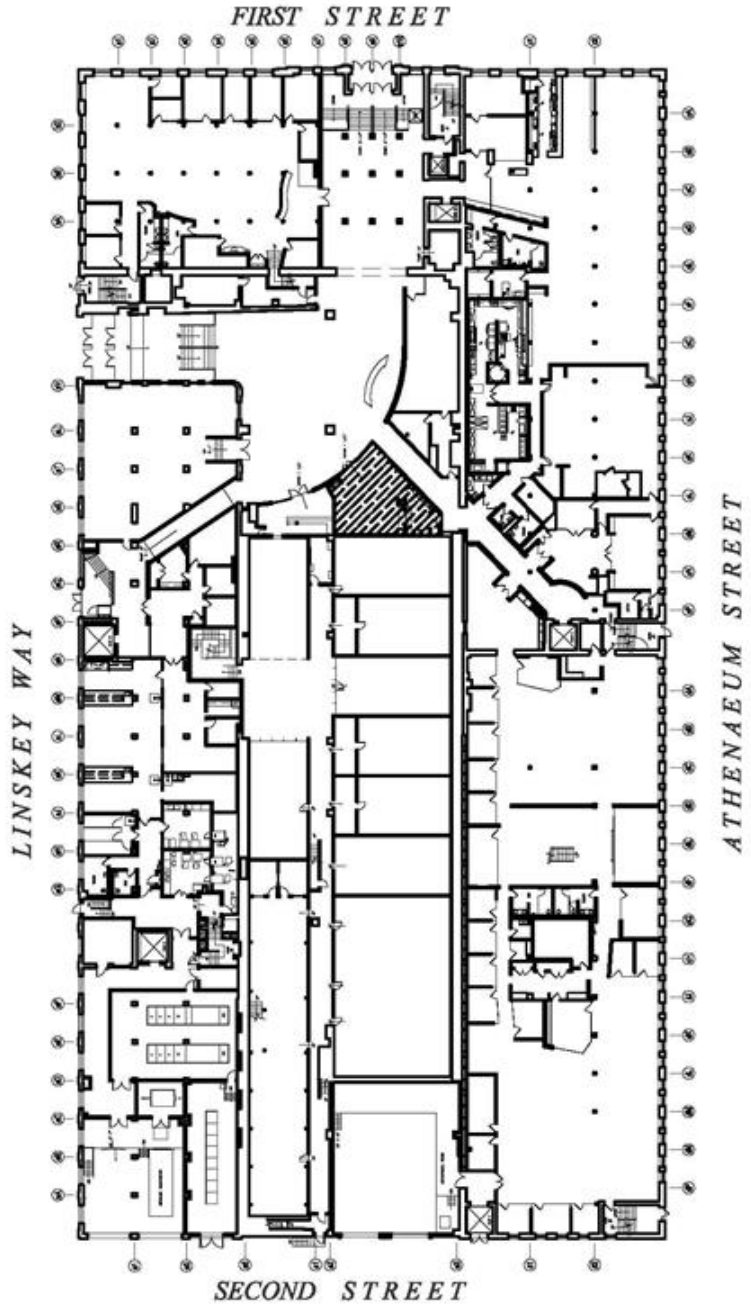


FLOOR 3

– THIRD FLOOR SIXTH EXPANSION PREMISES – 29,352 RSF

SAREPTA THERAPEUTICS

215 FIRST STREET
CAMBRIDGE, MASSACHUSETTS

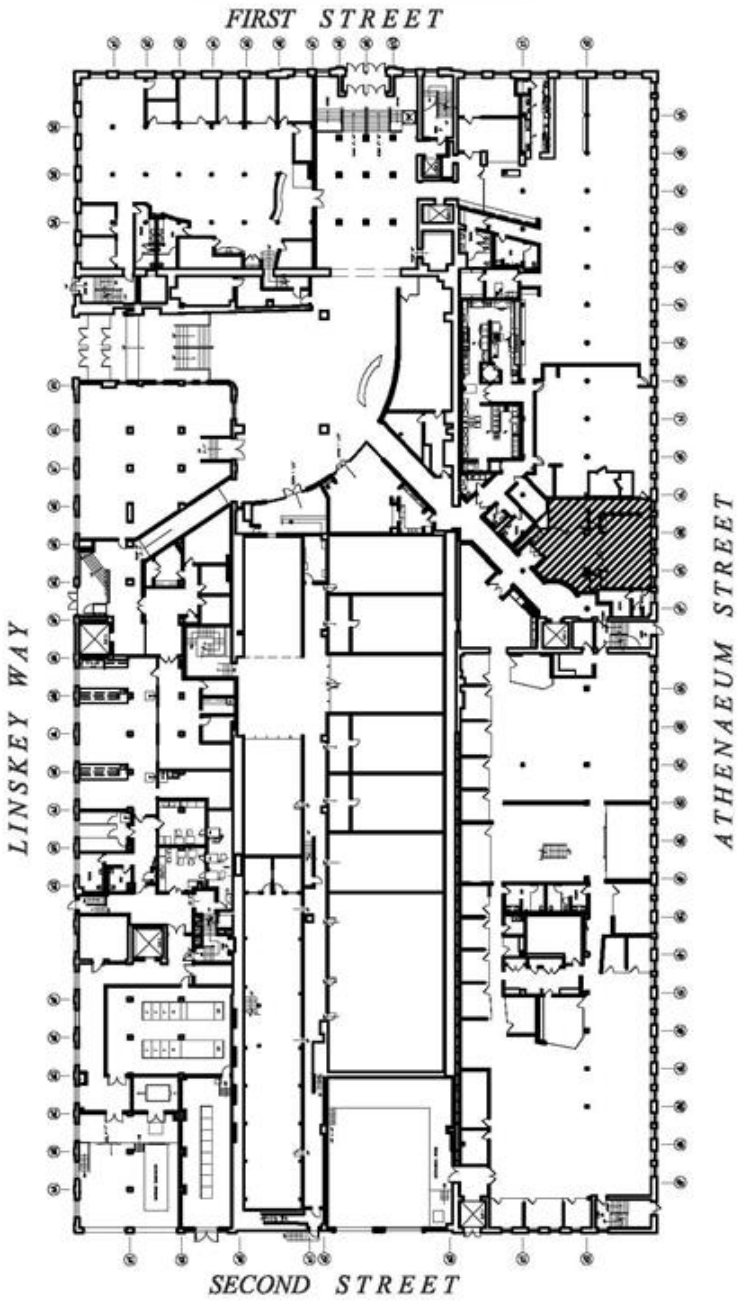


FLOOR 1

– FIRST FLOOR SIXTH EXPANSION PREMISES – 883 RSF

SAREPTA THERAPEUTICS

215 FIRST STREET
CAMBRIDGE, MASSACHUSETTS



FLOOR 1

– FIRST FLOOR SEVENTH EXPANSION PREMISES – 2,810 RSF

SAREPTA THERAPEUTICS

Exhibit B

Intentionally Omitted

Exhibit C

Work Letter

THIS WORK LETTER (this "**Work Letter**") is incorporated into that certain Lease Agreement dated as of June 25, 2013, as amended by that certain First Amendment to Lease dated as of November 13, 2013, as further amended by that certain Second Amendment to Lease dated as of February 18, 2014, as further amended by that certain Third Amendment to Lease dated as of July 31, 2014, as further amended by that certain Fourth Amendment to Lease dated as of August 28, 2014, and as further amended by that certain Fifth Amendment to Lease dated as of November 7, 2014, and as further amended by that certain Sixth Amendment to Lease dated as of November 30, 2016 and as further amended by that certain Seventh Amendment to Lease dated of even date herewith (as amended, the "**Lease**") by and between **ARE-MA REGION NO. 38, LLC**, a Delaware limited liability company ("**Landlord**"), and **SAREPTA THERAPEUTICS, INC.**, a Delaware corporation ("**Tenant**"). Any initially capitalized terms used but not defined herein shall have the meanings given them in the Lease.

1. **General Requirements**.

(a) **Tenant's Authorized Representative**. Tenant designates Robert Fay ("**Tenant's Representative**") as the only person authorized to act for Tenant pursuant to this Work Letter. Landlord shall not be obligated to respond to or act upon any request, approval, inquiry or other communication ("**Communication**") from or on behalf of Tenant in connection with this Work Letter unless such Communication is in writing from Tenant's Representative. Tenant may change Tenant's Representative at any time upon not less than 5 business days advance written notice to Landlord.

(b) **Landlord's Authorized Representative**. Landlord designates Jeff McComish and Tom Bryte (either such individual acting alone, "**Landlord's Representative**") as the only persons authorized to act for Landlord pursuant to this Work Letter. Tenant shall not be obligated to respond to or act upon any request, approval, inquiry or other Communication from or on behalf of Landlord in connection with this Work Letter unless such Communication is in writing from Landlord's Representative. Landlord may change either Landlord's Representative at any time upon not less than 5 business days advance written notice to Tenant.

(c) **Architects, Consultants and Contractors**. Landlord and Tenant hereby acknowledge and agree that the architect (the "**TI Architect**") for the Seventh Amendment Tenant Improvements (as defined in Section 2(a) below), the general contractor and any subcontractors for the Seventh Amendment Tenant Improvements shall be selected by Tenant, subject to Landlord's approval, which approval shall not be unreasonably withheld. Landlord consents to Tenant's selection of Ralph E. Dineen Architect as the TI Architect, to Timberline Construction Corp as the general contractor, and DPS Engineering as the engineer for the Seventh Amendment Tenant Improvements. Landlord shall be named a third party beneficiary of any contract entered into by Tenant with the TI Architect, any consultant, any contractor or any subcontractor, and of any warranty made by any contractor or any subcontractor.

2. **Seventh Amendment Tenant Improvements**.

(a) **Seventh Amendment Tenant Improvements Defined**. As used herein, "**Seventh Amendment Tenant Improvements**" shall mean all improvements to the Premises (including, without limitation, the Sixth Expansion Premises and the Seventh Expansion Premises) desired by Tenant of a fixed and permanent nature, which Seventh Amendment Tenant Improvements shall include, without limitation, the Staircase Improvements, the Restroom Improvements and the Base Building Improvements (all as defined in Section 5(b) below) and the conversion of the existing Building Systems controls in the Fourth Floor Sixth Expansion Premises and the Third Floor Sixth Expansion Premises to Johnson Controls Metasys systems. Landlord hereby approves of the conceptual plans for the Seventh Amendment Tenant Improvements as set forth on **Schedule 1** attached to this Work Letter. Notwithstanding anything to the

contrary contained in the Lease including, without limitation, the Seventh Amendment, other than funding the TI Allowance, the Restroom Allowance, the Staircase Allowance and the Base Building Allowance (all as defined in Section 5(b) below) as provided herein, Landlord shall not have any obligation whatsoever with respect to paying for the Seventh Amendment Tenant Improvements or otherwise finishing of the Sixth Expansion Premises or Seventh Expansion Premises for Tenant's use and occupancy.

Notwithstanding anything to the contrary in the Lease or the Seventh Amendment, Landlord shall cause, at Landlord's sole cost and expense (provided that nothing herein shall preclude Landlord from pursuing any prior tenants or third parties for reimbursement of such costs), the remediation, in a manner acceptable to Landlord in its reasonable discretion and otherwise in compliance with Legal Requirements, of Hazardous Materials not caused by Tenant or any Tenant Party requiring remediation pursuant to Legal Requirements discovered in the Sixth Expansion Premises or the Seventh Expansion Premises during the construction of the Seventh Amendment Tenant Improvements.

(b) **Tenant's Space Plans**. Tenant shall deliver to Landlord schematic drawings and outline specifications (the "**TI Design Drawings**") detailing Tenant's requirements for the Seventh Amendment Tenant Improvements. Not more than five (5) days thereafter, Landlord shall deliver to Tenant the written objections, questions or comments of Landlord with regard to the TI Design Drawings. Tenant shall cause the TI Design Drawings to be revised to address such written comments and shall resubmit said drawings to Landlord for approval. Such process shall continue until Landlord has approved the TI Design Drawings. Landlord's approval of the TI Design Drawings shall not be unreasonably withheld, conditioned or delayed.

(c) **Working Drawings**. Within a reasonable period following the approval of the TI Design Drawings by Landlord, Tenant shall cause the TI Architect to prepare and deliver to Landlord for review and comment construction plans, specifications and drawings for the Seventh Amendment Tenant Improvements ("**TI Construction Drawings**"), which TI Construction Drawings shall be prepared substantially in accordance with the TI Design Drawings. Tenant shall be solely responsible for ensuring that the TI Construction Drawings reflect Tenant's requirements for the Seventh Amendment Tenant Improvements. Landlord shall deliver its written comments on the TI Construction Drawings to Tenant not later than five (5) business days after Landlord's receipt of the same; provided, however, that Landlord may not disapprove any matter that is consistent with the TI Design Drawings. Tenant and the TI Architect shall consider all such comments in good faith and shall notify Landlord how Tenant proposes to respond to such comments. Any disputes in connection with such comments shall be resolved in accordance with Section 2(d) hereof. Provided that the design reflected in the TI Construction Drawings is consistent with the TI Design Drawings, Landlord shall approve the TI Construction Drawings submitted by Tenant. Once approved by Landlord, subject to the provisions of Section 4 below, Tenant shall not materially modify the TI Construction Drawings except as may be reasonably required in connection with the issuance of the TI Permit (as defined in Section 3(a) below). Landlord's approval of the TI Construction Drawings shall not be unreasonably, withheld, conditioned or delayed.

(d) **Approval and Completion**. If any dispute regarding the design of the Seventh Amendment Tenant Improvements is not settled within 10 business days after notice of such dispute is delivered by one party to the other, Tenant may make the final decision regarding the design of the Seventh Amendment Tenant Improvements, provided (i) Tenant acts reasonably and such final decision is either consistent with or a compromise between Landlord's and Tenant's positions with respect to such dispute, (ii) that all costs and expenses resulting from any such decision by Tenant shall be payable out of the TI Fund (as defined in Section 5(d) below), and (iii) Tenant's decision will not affect the base Building, structural components of the Building or any Building Systems (in which case Landlord shall make the final decision). Any changes to the TI Construction Drawings following Landlord's and Tenant's approval of same requested by Tenant shall be processed as provided in Section 4 hereof.

3. **Performance of the Seventh Amendment Tenant Improvements**.

(a) **Commencement and Permitting of the Seventh Amendment Tenant Improvements**. Tenant shall not commence construction of the Seventh Amendment Tenant Improvements prior to obtaining and delivering to Landlord a building permit (the "**TI Permit**") authorizing the construction of the Seventh Amendment Tenant Improvements consistent with the TI Construction Drawings approved by Landlord. The cost of obtaining the TI Permit shall be payable from the TI Fund. Landlord shall assist Tenant in obtaining the TI Permit. Prior to the commencement of the Seventh Amendment Tenant Improvements, Tenant shall deliver to Landlord a copy of any contract with Tenant's contractors (including the TI Architect), and certificates of insurance from any contractor performing any part of the Tenant Improvement evidencing industry standard commercial general liability, automotive liability, "builder's risk", and workers' compensation insurance. Tenant shall cause the general contractor to provide a certificate of insurance naming Landlord, Alexandria Real Estate Equities, Inc., and Landlord's lender (if any) as additional insureds for the general contractor's liability coverages required above.

(b) **Selection of Materials, Etc**. Where more than one type of material or structure is indicated on the TI Construction Drawings approved by Tenant and Landlord, the option will be within Tenant's reasonable discretion if the matter concerns the Seventh Amendment Tenant Improvements, and within Landlord's sole and absolute subjective discretion if the matter concerns the structural components of the Building or any Building System.

(c) **Tenant Liability**. Tenant shall be responsible for correcting any deficiencies or defects in the Seventh Amendment Tenant Improvements.

(d) **Substantial Completion**. Tenant shall substantially complete or cause to be substantially completed the Seventh Amendment Tenant Improvements in a good and workmanlike manner, in accordance with the TI Permit subject, in each case, to Minor Variations and normal "punch list" items of a non-material nature which do not interfere with the use of the Premises ("**Substantial Completion**" or "**Substantially Complete**"). Upon Substantial Completion of the Seventh Amendment Tenant Improvements, Tenant shall require the TI Architect and the general contractor to execute and deliver, for the benefit of Tenant and Landlord, a Certificate of Substantial Completion in the form of the American Institute of Architects ("**AIA**") document G704. For purposes of this Work Letter, "**Minor Variations**" shall mean any modifications reasonably required: (i) to comply with all applicable Legal Requirements and/or to obtain or to comply with any required permit (including the TI Permit); (ii) to comport with good design, engineering, and construction practices which are not material; or (iii) to make reasonable adjustments for field deviations or conditions encountered during the construction of the Seventh Amendment Tenant Improvements.

4. **Changes**. Any changes requested by Tenant to the Seventh Amendment Tenant Improvements after the delivery and approval by Landlord of the TI Design Drawings, shall be requested and instituted in accordance with the provisions of this Section 4 and shall be subject to the written approval of Landlord, which approval shall not be unreasonably withheld, conditioned or delayed.

(a) **Tenant's Right to Request Changes**. If Tenant shall request changes ("**Changes**"), Tenant shall request such Changes by notifying Landlord in writing in substantially the same form as the AIA standard change order form (a "**Change Request**"), which Change Request shall detail the nature and extent of any such Change. Such Change Request must be signed by Tenant's Representative. Landlord shall review and approve or disapprove such Change Request within five (5) business days thereafter, provided that Landlord's approval shall not be unreasonably withheld, conditioned or delayed.

(b) **Implementation of Changes**. If Landlord approves such Change, Tenant may cause the approved Change to be instituted. If any TI Permit modification or change is required as a result of such Change, Tenant shall promptly provide Landlord with a copy of such TI Permit modification or change.

5. **Costs**.

(a) **Budget For Seventh Amendment Tenant Improvements**. Before the commencement of construction of the Seventh Amendment Tenant Improvements, Tenant shall obtain a detailed breakdown, by trade, of the costs incurred or that will be incurred, in connection with the design and construction of the Seventh Amendment Tenant Improvements (the "**Budget**"), and deliver a copy of the Budget to Landlord for Landlord's approval, which shall not be unreasonably withheld, conditioned or delayed. The Budget shall be based upon the TI Construction Drawings approved by Landlord.

(b) **TI Allowance**. Landlord shall provide to Tenant a tenant improvement allowance ("**TI Allowance**") of $5,208,850 in the aggregate. The TI Allowance shall be disbursed in accordance with this Work Letter.

The TI Allowance shall be disbursed in accordance with this Work Letter. Except as otherwise expressly provided in this Section 5, Tenant shall have no right to the use or benefit (including any reduction to Base Rent) of any portion of the TI Allowance not required for the construction of (i) the Seventh Amendment Tenant Improvements described in the TI Construction Drawings approved pursuant to Section 2(d) or other Alterations in the Premises approved by Landlord ("**Seventh Amendment Alterations**"), or (ii) any Changes pursuant to Section 4. Tenant shall have no right to any portion of the TI Allowance that is not disbursed before the date that is 21 months after the Sixth Expansion Premises Commencement Date. Notwithstanding anything to the contrary contained herein, any unused portion of the TI Allowance may be used by Tenant for either the Staircase Improvements, the Base Building Improvements or the Restroom Improvements.

In addition to the TI Allowance, Landlord shall provide to Tenant an allowance for the costs incurred by Tenant, of $800,000 (the "**Restroom Allowance**") for the renovation of the restrooms located in the Fourth Floor Sixth Expansion Premises and the Third Floor Sixth Expansion Premises (the "**Restroom Improvements**). Tenant shall be responsible for the cost of the Restroom Improvements in excess of the Restroom Allowance. Notwithstanding anything to the contrary contained herein, any unused portion of the Restroom Allowance may be used by Tenant for either the Staircase Improvements, the Base Building Improvements or the Seventh Amendment Tenant Improvements.

Landlord shall also provide Tenant with an allowance, in the amount of $150,000 (the "**Staircase Allowance**"), for costs incurred by Tenant for the installation of a staircase interconnecting the Fourth Floor Sixth Expansion Premises and the Third Floor Sixth Expansion Premises ("**Staircase Improvements**"), which Staircase Allowance shall, to the extent used, result in Additional Rent as set forth in Section 9 of the Seventh Amendment. Tenant shall be responsible for the cost of the Staircase Improvements in excess of the Staircase Allowance. Notwithstanding anything to the contrary contained herein, any unused portion of the Staircase Allowance may be used by Tenant for either the Restroom Improvements, the Base Building Improvements or the Seventh Amendment Tenant Improvements.

Landlord shall also provide Tenant with an allowance, in the amount of $4,250,000 (the "**Base Building Allowance**"), for costs incurred by Tenant to be comprised of (i) $1,000,000 for the construction of certain improvements to the Base Building affecting the Existing Premises in accordance with the plans approved by Landlord, which approval shall not be unreasonably withheld, conditioned or delayed, including, without limitation, improvements to the boilers, controls and HVAC distribution affecting the Existing Premises, (ii) $750,000 for costs incurred for the HVAC system to be installed in the Third Floor Sixth Expansion Premises, (iii) $750,000 for costs incurred for the HVAC system to be installed in the Fourth Floor Sixth Expansion Premises, and (iv) $1,750,000 for costs incurred in connection with the purchase and installation of skylights to be installed in the Fourth Floor Sixth Expansion Premises as shown on the roof plan attached hereto as **Schedule 2**, the approximately size and location of which has been agreed upon y Landlord and Tenant (collectively, "**Base Building Improvements**"). Landlord shall reasonably assist Tenant, at no cost to Landlord, in obtaining any permits required for the Base Building Improvements. Tenant shall be responsible for the cost of the Base Building Improvements in excess of the Base Building

Allowance. Any unused portion of the Base Building Allowance may be used by Tenant for either the Restroom Improvements, the Staircase Improvements or the Seventh Amendment Tenant Improvements.

(c) **Costs Includable in TI Fund**. The TI Fund shall be used solely for the payment of design, permits and construction costs in connection with the construction of the Seventh Amendment Tenant Improvements or the Seventh Amendment Alterations, including, without limitation, the cost of electrical power and other utilities used in connection with the construction of the Seventh Amendment Tenant Improvements, the cost of preparing the TI Design Drawings and the TI Construction Drawings, all costs set forth in the Budget and the cost of Changes (collectively, "**TI Costs**"). Notwithstanding anything to the contrary contained herein, other than as provided in the immediately following sentence, the TI Fund shall not be used to purchase any furniture, personal property or other trade fixtures or equipment, including, but not be limited to, Tenant's voice or data cabling, non-ducted biological safety cabinets and other scientific equipment not incorporated into the Seventh Amendment Tenant Improvements. Notwithstanding anything to the contrary contained in the Lease, Landlord shall not be entitled to a construction management fee, oversight fee or supervisions fee in connection with the Seventh Amendment Tenant Improvements or the Seventh Amendment Alterations. In the event that Landlord incurs actual out-of—pocket costs in connection with Landlord's review of plans and specifications for the Seventh Amendment Tenant Improvements and the Seventh Amendment Alterations, the maximum amount that Tenant shall be required to reimburse Landlord for such review shall be $10,000.00.

(d) **Excess TI Costs**. Landlord shall have no obligation to bear any portion of the cost of any of the Seventh Amendment Tenant Improvements except to the extent of the TI Allowance (and the Restroom Allowance, the Staircase Allowance and the Base Building Allowance, as applicable). If at any time and from time-to-time, the remaining TI Costs under the Budget exceed the remaining unexpended TI Allowance (and the Restroom Allowance, the Staircase Allowance and the Base Building Allowance, as applicable) (collectively, "**Excess TI Costs**"), monthly disbursements of the TI Allowance shall be made in the proportion that the remaining TI Allowance bears to the outstanding TI Costs under the Budget, and Tenant shall fund the balance of each such monthly draw. For purposes of any litigation instituted with regard to such amounts, those amounts required to be paid by Tenant will be deemed Rent under the Lease. The TI Allowance, the Restroom Allowance, the Staircase Allowance, the Base Building Allowance and Excess TI Costs are herein referred to as the "**TI Fund**." Notwithstanding anything to the contrary set forth in this Section 5(d), Tenant shall be fully and solely liable for TI Costs and the cost of Minor Variations in excess of the TI Allowance.

(e) **Payment for TI Costs**. During the course of design and construction of the Seventh Amendment Tenant Improvements, subject to the terms of Section 5(d), Landlord shall reimburse Tenant for TI Costs once a month against a draw request in Landlord's standard form, containing evidence of payment of such TI Costs by Tenant and such certifications, lien waivers (including a conditional lien release for each progress payment and unconditional lien releases for the prior month's progress payments), inspection reports and other matters as Landlord customarily obtains, to the extent of Landlord's approval thereof for payment, no later than 30 days following receipt of such draw request. Upon completion of the Seventh Amendment Tenant Improvements (and prior to any final disbursement of the TI Fund), Tenant shall deliver to Landlord: (i) sworn statements setting forth the names of all contractors and first tier subcontractors who did the work and final, unconditional lien waivers from all such contractors and first tier subcontractors; (ii) as-built plans (one copy in print format and two copies in electronic CAD format) for such Seventh Amendment Tenant Improvements; (iii) a certification of substantial completion in Form AIA G704, (iv) a certificate of occupancy for the Premises; and (v) copies of all operation and maintenance manuals and warranties affecting the Premises.

6. **Miscellaneous**.

(a) **Consents**. Whenever consent or approval of either party is required under this Work Letter, that party shall not unreasonably withhold, condition or delay such consent or approval, except as may be expressly set forth herein to the contrary.

(b) **Modification**. No modification, waiver or amendment of this Work Letter or of any of its conditions or provisions shall be binding upon Landlord or Tenant unless in writing signed by Landlord and Tenant.

(c) **No Default Funding**. In no event shall Landlord have any obligation to fund any portion of the TI Allowance, the Restroom Allowance or the Staircase Allowance during any period that Tenant is in Default under the Lease.

Schedule 1

Conceptual Plans

RICHMOND

Scope of Work

Alexandria- 3rd/4th Floor C/S Scope	**Project # TBD**
215 First Street, Cambridge MA	January 08, 2018

1. **Architectural**

   - Misc steel for shaft related work
   - Provide new structural steel for new rooftop units
   - New toilet cores for the 4th floor to accommodate 200 people
   - Refurbish existing toilet rooms on the 3rd floor
   - Demolition on the 3rd floor office area
   - 4th floor duct shaft for new 3rd floor rooftop unit distribution

2. **Fire Protection**

   - Rework sprinkler heads on the 3rd floor for upright configuration
   - Provide new sprinklers in electric and mechanical rooms
   - Provide new sprinkler related work for all new common areas

3. **Plumbing**

   - Rework existing gas piping to serve new HVAC boilers
   - New domestic cold water line for tenant use on the 3rd/4th floors, 2 inch valve/cap connection
   - Domestic cold water to new toilet cores
   - Provide new electric hot water heater above ceiling for new toilet cores, with drain pan and water bug, to shut off cold water if water is present
   - Domestic waste/vent for new toilet cores
   - Rework existing toilet cores to include new fixtures
   - New cooler drinking fountain for the 3rd/4th floors
   - New janitors closet for the 4th floor
   - Refurbish 3rd floor janitors closet

4. **Electrical**

   - Re-use existing base building electric rooms
   - Re-use existing electric room 441
   - Panels DPM2 and DP44 located in electric room 411 need to be relocated to a new electric room
   - Route new power from main electric room on the 2nd floor, or provide j-box at current electric room location and route new wires to new electric room location
   - Panel DPM2 will have to be phased or new panel installed to accommodate existing equipment to remain operational
   - New power distribution to new HVAC and Plumbing equipment
   - New lighting and convenience outlets in common areas and toilet cores
   - Distribution of fire alarm and life safety as required to common areas
   - Card access excluded

- Security excluded
- Tel/data excluded
- AV excluded

SEE ADDITIONAL DOCUMENT FOR HVAC SCOPE OF WORK / BASIS OF DESIGN..

BASIS OF DESIGN

Date: March 28, 2018
Subject: 215 First St., Cambridge, HVAC Systems Basis of Design

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The intent of the below HVAC scope of work is to capture the base building alterations needed to be able to renovate the 3rd and 4th floors for a new office tenant.

HVAC

1. Remove the two existing Trane Intellipak package rooftop HVAC units (one 50 ton, one 75 ton) from the structural framing on the roof. Remove the existing McQuay 50 ton package rooftop HVAC unit from the structural framing on the roof. Remove the four Carrier packaged rooftop HVAC unit (2 @ 7.5 ton, 2 @ 12.5 ton) and roof curbs from the roof. Coordinate with plumbing and electrical contractors to remove the gas piping and power feeds from these units. Coordinate with roofing contractor to patch and repair roofing where the roof curbs are removed.

2. Provide one new nominal 90 Ton Trane Packaged Air-Cooled Dx Intellipak Rooftop Unit on the west side structural framing (in place of the removed 75 ton unit). Provide one new nominal 50 Ton Trane Packaged Air-Cooled Dx Intellipak Rooftop Unit on the east side structural framing (in place of the removed 50 ton unit). Provide one new nominal 50 Ton Trane Packaged Air-Cooled Dx Intellipak Rooftop Unit on the south side structural framing (in place of the removed 50 ton McQuay unit). to serve the 4th floor office area. Units shall be mounted on vibration isolation rail on dunnage, with return/exhaust fan with 100% Economizer, VFD (VAV) control and morning warm-up via reheat boxes and recirculation. Provide internal control unit with BACnet communications for interface with the building control system. Connect existing supply and return ducts in each location to the new unit supply and return connections with flexible duct connectors and new fire/smoke damper and smoke detectors.

3. Provide one new nominal 50 Ton Trane Packaged Air-Cooled Dx Intellipak Rooftop Unit on the west side structural framing (in the open space beside the new 90 ton unit). Units shall be mounted on vibration isolation rail on dunnage, with return/exhaust fan with 100% Economizer, VFD (VAV) control and morning warm-up via reheat boxes and recirculation. Provide internal control unit with BACnet communications for interface with the building control system.

4. Provide new SA/RA Ductwork riser for new 50 Ton Packaged unit down through roof to the 3rd floor. SA duct shall be 60 x 24 thru roof, with RA Duct shall be 60 x 36. Provide SA duct with fire/smoke dampers and detector. Provide supply and return duct sound attenuators.

Lexington, MA
24 Hartwell Avenue
3rd Floor
Lexington, MA 02421
T 781-372-3000

Cambridge, MA
700 Technology Square
Suite 402
Cambridge, MA 02139
T 781-372-3000

Atlanta, GA
3700 Mansell Road
Suite 200
Alpharetta, GA 30022
T 770-992-8585

Washington, DC
3000 Wilson Boulevard
Suite 210
Arlington, VA 22201
T 571-451-1940

5. All Supply air duct from RTU down through the roof shall be rated for 4.0" pressure construction. All Return air duct from RTU down through the roof shall be rated for 2.0" pressure construction.

6. Provide new DDC automatic controls for all new equipment. The existing server and network will remain and be reused. Provide BacNet interface with packaged equipment controllers for the new rooftop units. Provide new panels as required as an extension of the existing building controls, update graphics and provide similar points as utilized by the existing equipment.

7. Provide new duct with rigid board duct insulation, and wrap with roofing membrane and seal weathertight. Replace existing supply and return duct insulation with new insulation and roofing membrane at the removed and replaced 50 ton McQuay unit, from the unit connection to the roof pentrations.

8. Provide rigging of new rooftop units and removal of existing units.

9. Provide three new 1500 MBH gas fired condensing hot water boilers with 90 GPM primary boiler

pumps per boiler and (2) 300 GPM hot water loop pumps, on VFD control with differential bypass control.

10. Remove the existing Hydrotherm boilers in the second floor mechanical room, in a phased demo to maintain partial heating loop operation.

11. Provide new boiler direct vent double wall flues and insulated intake ducts, air separator, expansion tank and hot water loop accessories.

12. Provide new main HW piping loop to be 4 inch, connecting the existing distribution and new connection for 3 inch piping risers, and 2 ½ valve cap connections for the third and fourth floors for tenant use. Insulate new HW piping with 2" thick fiberglass pipe insulation faced with ASJ.

13. Provide testing and balancing of new HVAC equipment.

End of Basis of Design

\\c-fs2\g_drive\Projects\2018-Boston-Cam-Rep\M0650-005.20\Correspondence\Scope of Work\2018-03-28 HVAC Basis of Design.docx

Schedule 2

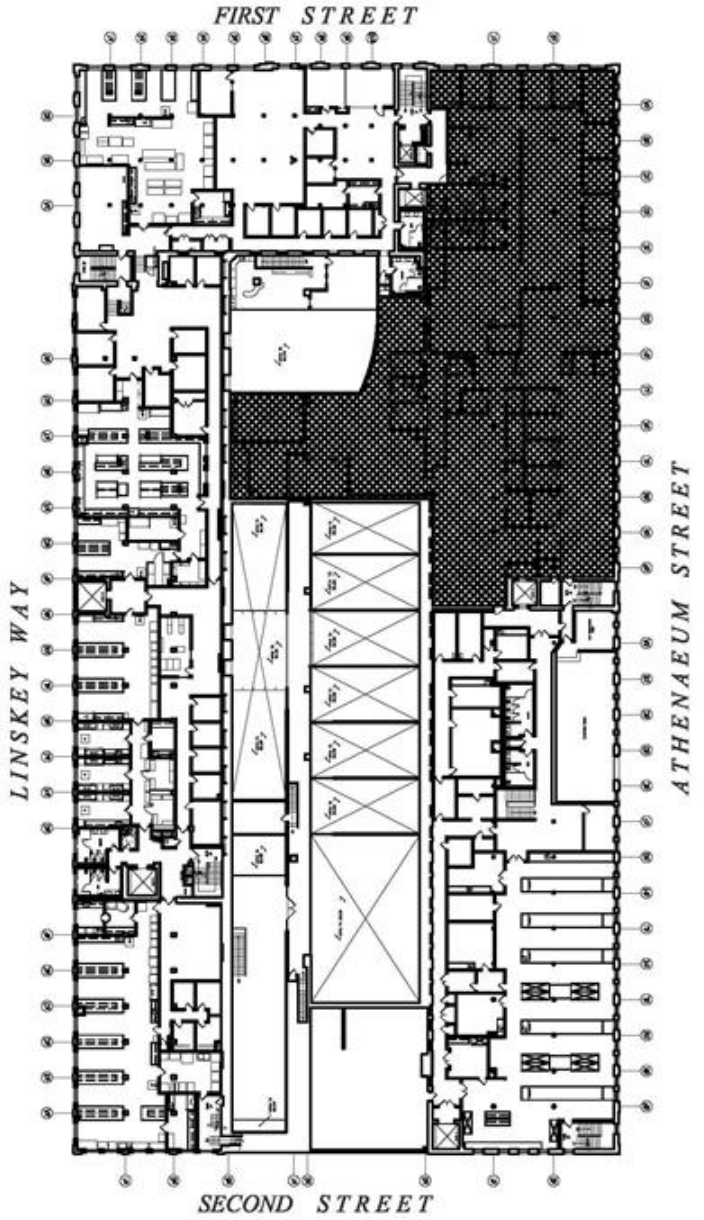
Roof Plans

Exhibit D

Available Space

215 FIRST STREET
CAMBRIDGE, MASSACHUSETTS

EXHIBIT D



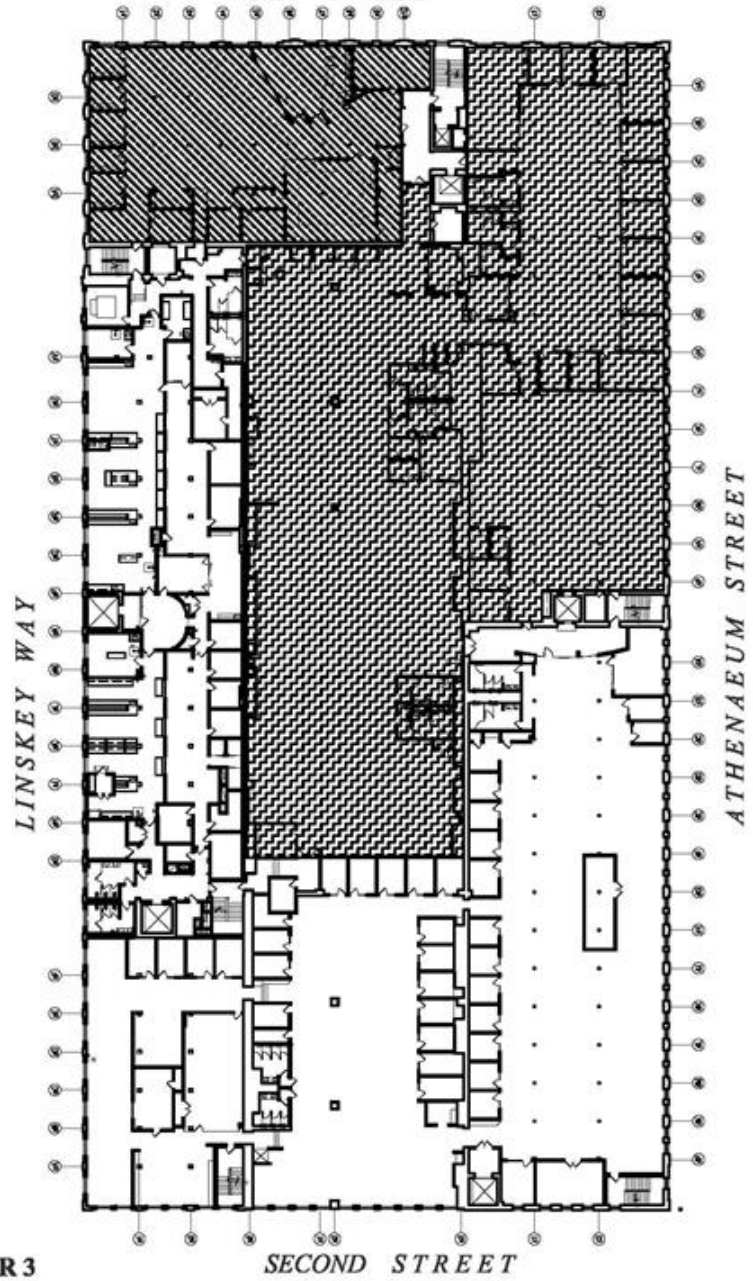
FLOOR 2

– EXPANSION RIGHT/ AVAILABLE SPACE – 18,562 RSF

SAREPTA THERAPEUTICS

215 FIRST STREET
CAMBRIDGE, MASSACHUSETTS

EXHIBIT D



FLOOR 3

– EXPANSION RIGHT/ AVAILABLE SPACE – 32,876 RSF

– EXPANSION RIGHT/ AVAILABLE SPACE – 8,875 RSF

SAREPTA THERAPEUTICS

Exhibit E

Base Rent Schedule

Amendment Definition:		Original Premises	Original Premises	Expansion Premises	Second Expansion Premises	Third Expansion Premises	Fourth Expansion Premises	Fifth Expansion Premises	Sixth Expansion Premises	First Floor Expansion Premises	Third Floor Sixth Expansion Premises	Lower Level Sixth Expansion Premises	Seventh Expansion Premises
Annual increase		2%	2%	2%	3%	3%	2%	2%	2%	2%	2%	3%	2%
Use		Office/Lab	Office	Office	Lower Level Office	Lower Level Office	Office	Office	Office	Office (Reception)	Office	Lower Level Office	Office
Floor		1st/2nd Floor	4th Floor	4th Floor	Lower Level	Lower Level	1st/2nd Floor	1st Floor	4th Floor	1st Floor	3rd Floor	Lower Level	1st Floor
Year	**Date**	32,314	14,062	15,077	4,031	4,445	7,461	11,069	28,258	883	29,352	2,395	2,810
1	4/1/2018	$ 73.00	$ 53.00	$ 53.00	$ 30.00	$ 30.00	$ 53.00	$ 53.00	$ 43.00	$ 53.00	$ 53.00	$ 30.00	$ 53.00
2	2/1/2019	$ 74.46	$ 54.06	$ 54.06	$ 30.90	$ 30.90	$ 54.06	$ 54.06	$ 43.86	$ 54.06	$ 54.06	$ 30.90	$ 54.06
3	2/1/2020	$ 75.95	$ 55.14	$ 55.14	$ 31.83	$ 31.83	$ 55.14	$ 55.14	$ 44.74	$ 55.14	$ 55.14	$ 31.83	$ 55.14
4	2/1/2021	$ 77.47	$ 56.24	$ 56.24	$ 32.78	$ 32.78	$ 56.24	$ 56.24	$ 45.63	$ 56.24	$ 56.24	$ 32.78	$ 56.24
5	2/1/2022	$ 79.02	$ 57.37	$ 57.37	$ 33.77	$ 33.77	$ 57.37	$ 57.37	$ 46.54	$ 57.37	$ 57.37	$ 33.77	$ 57.37
6	2/1/2023	$ 80.60	$ 58.52	$ 58.52	$ 34.78	$ 34.78	$ 58.52	$ 58.52	$ 47.48	$ 58.52	$ 58.52	$ 34.78	$ 58.52
7	2/1/2024	$ 82.21	$ 59.69	$ 59.69	$ 35.82	$ 35.82	$ 59.69	$ 59.69	$ 48.42	$ 59.69	$ 59.69	$ 35.82	$ 59.69
7*	2/1/2025	$ 83.85	$ 60.88	$ 60.88	$ 36.90	$ 36.90	$ 60.88	$ 60.88	$ 49.39	$ 60.88	$ 60.88	$ 36.90	$ 60.88
Rent Commencement Date		2/1/2021	2/1/2021	2/1/2021	2/1/2021	2/1/2021	2/1/2021	2/1/2021	10/1/2018	7/1/2018	7/1/2018	10/1/2018	2/1/2019 (Est.)
New Rent Paying Term (yrs.)		4.67	4.67	4.67	4.67	4.67	4.67	4.67	7.00	7.25	7.25	7.00	6.67
Lease Expiration Date		9/30/2025	9/30/2025	9/30/2025	9/30/2025	9/30/2025	9/30/2025	9/30/2025	9/30/2025	9/30/2025	9/30/2025	9/30/2025	9/30/2025

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.

May 3, 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.

May 3, 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 March 31, 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.

May 3, 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 March 31, 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.

May 3, 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.