Sarepta Therapeutics, Inc.
Form 10-Q
November 07, 2016

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 September 30, 2016

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from to

Commission file number 001-14895

SAREPTA THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	93-0797222
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification 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, or a smaller reporting company. See the definitions of “large accelerated filer,” “accelerated filer” and “smaller reporting company” in Rule 12b-2 of the Exchange Act (Check one):

Large accelerated filer Accelerated filer

Non-accelerated filer (Do not check if a smaller reporting company) Smaller Reporting Company

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the 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	54,590,380
(Class)	(Outstanding as of November 3, 2016)

SAREPTA THERAPEUTICS, INC.

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PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

(unaudited, in thousands, except shares and per share amounts)

	As of September 30, 2016	As of December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	$ 395,140	$ 80,304
Short-term investments	—	112,187
Accounts receivable	3,986	3,977
Restricted investment	10,695	10,695
Inventory	2,921	—
Other current assets	22,002	17,380
Total current assets	434,744	224,543
Restricted cash and investments	784	783
Property and equipment, net of accumulated depreciation of $28,426 and $24,594 as of September 30, 2016 and December 31, 2015, respectively	35,620	37,344
Intangible assets, net of accumulated amortization of $3,054 and $2,620 as of September 30, 2016 and December 31, 2015, respectively	8,111	6,642

Other non-current assets	8,051	4,470
Total assets	$ 487,310	$ 273,782
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 20,684	$ 20,234
Accrued expenses	29,539	29,053
Current portion of long-term debt	10,107	5,936
Current portion of notes payable	—	2,493
Deferred revenue	3,303	3,303
Other current liabilities	1,300	1,275
Total current liabilities	64,933	62,294
Long-term debt	8,491	14,969
Deferred rent and other	5,262	6,172
Total liabilities	78,686	83,435
Commitments and contingencies (Note 12)		
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; 54,351,725 and 45,629,529 issued and outstanding at September 30, 2016 and December 31, 2015, respectively	5	5
Additional paid-in capital	1,486,487	1,089,508
Accumulated other comprehensive loss	—	(111)
Accumulated deficit	(1,077,868)	(899,055)
Total stockholders' equity	408,624	190,347
Total liabilities and stockholders' equity	$ 487,310	$ 273,782

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 September 30,		For the Nine Months Ended September 30,	
	2016	2015	2016	2015
Revenues	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	34,349	36,673	117,523	105,018
General and administrative	22,184	15,090	60,812	50,714
Total operating expenses	56,533	51,763	178,335	155,732
Operating loss	(56,533)	(51,763)	(178,335)	(155,732)
Other income (loss):				
Interest (expense) income and other, net	(209)	(176)	(478)	383
Total other (loss) income	(209)	(176)	(478)	383
Net loss	$ (56,742)	$ (51,939)	$ (178,813)	$ (155,349)
Other comprehensive income (loss):				
Unrealized (loss) gain on short-term securities - available-for-sale	(1)	18	111	94
Total other comprehensive (loss) income	(1)	18	111	94
Comprehensive loss	$ (56,743)	$ (51,921)	$ (178,702)	$ (155,255)
Net loss per share — basic and diluted	$ (1.18)	$ (1.25)	$ (3.83)	$ (3.75)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	48,254	41,565	46,709	41,416

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(unaudited, in thousands)

	For the Nine Months Ended September 30,	
	2016	2015
Cash flows from operating activities:		
Net loss	$ (178,813)	$ (155,349)
Adjustments to reconcile net loss to cash flows from operating activities:		
Depreciation and amortization	3,947	3,883
Amortization of premium on available-for-sale securities, loss from sale of available-for-sale securities and non-cash interest	473	805
Loss on abandonment of patents	45	180
Stock-based compensation	23,093	25,769
Non-cash restructuring expenses	504	—
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable	(9)	(317)
Net increase in inventory	(2,921)	—
Net (increase) decrease in other assets	(8,203)	9,963
Net decrease in accounts payable, accrued expenses, deferred revenue and other liabilities	(2,703)	(3,127)
Net cash used in operating activities	(164,587)	(118,193)
Cash flows from investing activities:		
Purchase of property and equipment	(2,427)	(2,316)
Purchase of intangible assets	(1,093)	(982)
Purchase of restricted investments	—	(10,695)
Purchase of available-for-sale securities	—	(49,632)
Sale and maturity of available-for-sale securities	112,101	141,854
Net cash provided by investing activities	108,581	78,229
Cash flows from financing activities:		
Proceeds from borrowings, net of debt issuance costs	—	19,601
Repayments of long-term debt and notes payable	(5,076)	(2,573)
Proceeds from sales of common stock	364,951	—
Proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program	10,967	5,204
Net cash provided by financing activities	370,842	22,232
Increase (decrease) in cash and cash equivalents	314,836	(17,732)
Cash and cash equivalents:		
Beginning of period	80,304	73,551

End of period	395,140	55,819
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 1,199	$ 359
Supplemental schedule of non-cash investing activities and financing activities:		
Shares withheld for taxes	$ 1,955	$ —
Accrual for debt issuance costs related to the senior secured term loan	$ 400	$ 400
Intangible assets included in accrued expenses	$ 1,230	$ 105
Accrual for offering costs related to the equity offerings	$ 222	$ 135
Property and equipment included in accrued expenses	$ —	$ 211
Capitalized interest	$ —	$ 99

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

1. BUSINESS AND BASIS OF PRESENTATION

Business

Sarepta Therapeutics, Inc. (together with its wholly-owned subsidiaries, “Sarepta” or the “Company”) is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. Applying its proprietary, highly-differentiated and innovative platform technologies, the Company is able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (“DMD”) drug candidates. On September 19, 2016, the United States Food and Drug Administration (“FDA”) granted accelerated approval for EXONDYS 51, indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 is studied in clinical trials under the name of eteplirsen and is marketed in the U.S. under the trademarked name of EXONDYS 51™ (eteplirsen) Injection.

Through September 30, 2016, the Company had not generated any revenue from product sales, and the Company may never generate substantial revenue from product sales. Even if sales of EXONDYS 51 generate substantial revenue, the Company is likely to continue to incur operating losses in the near term.

As of September 30, 2016, the Company had approximately $406.6 million of cash, cash equivalents and restricted cash and investments, consisting of $395.1 million of cash and cash equivalents and $11.5 million of restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments as of September 30, 2016 is sufficient to fund its current operational plan for the next twelve months, though it may pursue additional cash resources through public or private financings, sell its Priority Review Voucher, seek additional government funding and establish collaborations with or license its technology to other companies.

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 of America (“U.S. GAAP”), reflect the accounts of Sarepta Therapeutics, Inc. and its wholly-owned subsidiaries. All inter-company transactions between and among its consolidated subsidiaries have been eliminated. Management has determined that the Company operates in one segment: the development of pharmaceutical products. The information included in this quarterly report on Form 10-Q should be read in conjunction with the Company’s consolidated financial statements and the accompanying notes included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2015.

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 the valuation of stock-based awards, research and development expenses and income taxes.

2. SIGNIFICANT ACCOUNTING POLICIES AND RECENT ACCOUNTING PRONOUNCEMENTS

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, 2015. Related to the commercialization of EXONDYS 51, the Company updated its significant accounting policies as follows:

Revenue Recognition

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

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

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

The Company establishes reserves for various government rebate programs and co-payment assistance. Reserves established for these discounts and allowances are classified as either reductions of accounts receivable or a liability. These reserves are based on estimates of the amounts earned or to be claimed on the related sales.

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

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 upon regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. Drug products to 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 September 30, 2016 (in thousands)
Raw materials	$ 2,712
Finished goods	209
Total inventory	$ 2,921

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. As a result, the Company will record a charge to cost of sales to write down any unmarketable inventory to its estimated net realizable value.

Intangible Assets

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

The in-licensed right relates to the license agreement with the University of Western Australia ("UWA"). As a result of the FDA approval and the subsequent commercial sale of EXONDYS 51, as defined in the Amended and Restated UWA License Agreement (defined in Note 3), the Company was obligated to pay a $1.0 million sales milestone to UWA and, accordingly, has recorded an in-licensed right. The in-licensed right will be amortized on a straight-line basis over the remaining life of the related patent because the life of the related patent reflects the expected time period that the Company will benefit from the in-licensed right. The amortization of the in-licensed right will be recorded as cost of goods sold in the Company's consolidated statements of operations and comprehensive loss.

The following table summarizes the components of intangible assets for the period indicated:

	As of September 30, 2016 (in thousands)
Patent costs	$ 10,165
In-licensed right	1,000
Intangible assets, gross	$ 11,165
Less: accumulated amortization	(3,054)
Intangible assets, net	$ 8,111

There have not been any other material changes to the Company's accounting policies as of September 30, 2016.

Recent Accounting Pronouncements

In August 2016, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2016-15, "Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments". The amendments in this update clarify how certain cash receipts and cash payments are presented and classified in the statement of cash flows. ASU No. 2016-15 will be effective for fiscal years beginning after December 15, 2017, with early adoption permitted. As of September 30, 2016, the Company has not elected to early adopt this guidance and does not expect the adoption of this guidance to have any impact on its consolidated financial statements.

In March 2016, the FASB issued ASU No. 2016-09, "Improvements to Employee Share-Based Payment Accounting". The amendments in this update simplify several aspects of the accounting for share-based payment transactions, including income tax consequences, classification of awards as either equity or liabilities and classification on the statement of cash flows. ASU No. 2016-09 will be effective for fiscal years beginning after December 15, 2016, with early adoption permitted. As of September 30, 2016, the Company has not elected to early adopt this guidance but determined that the adoption of this standard will not have any impact on the Company's financial statements.

In February 2016, the FASB issued ASU No. 2016-02, "Leases (Topic 842)", which supersedes Topic 840, "Leases". Under the new guidance, a lessee should recognize assets and liabilities that arise from its leases and disclose qualitative and quantitative information about its leasing arrangements. ASU No. 2016-02 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted. As of September 30, 2016, 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 July 2015, the FASB issued ASU No. 2015-11, "Inventory (Topic 330): Simplifying the Measurement of Inventory". The new standard applies only to inventory for which cost is determined by methods other than last-in, first-out and the retail inventory method, which includes inventory that is measured using first-in, first-out or average cost. Inventory within the scope of this standard is required to be measured at the lower of cost and net realizable value. Net realizable value is the estimated selling prices in the ordinary course of business, less reasonably predictable costs of completion, disposal, and transportation. The new standard will be effective for fiscal years beginning after December 15, 2016. As of September 30, 2016, the Company has not elected to early adopt this guidance but determined that the adoption of this standard will not have any impact on the Company's financial statements.

In August 2014, the FASB issued ASU No. 2014-15, "Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern". This update requires an entity's management to evaluate whether there are conditions or events, considered in the aggregate, that raise substantial doubt about the entity's ability to continue as a going concern within one year after the date that the financial statements are issued or available to be issued and to provide related disclosures. ASU No. 2014-15 will be effective for the fiscal years beginning after December 15, 2016, with early adoption permitted. As of September 30, 2016, the Company has not elected to early adopt this guidance, and based on the Company's financial condition as of the date these financial statements were issued or available for issuance, the Company does not expect the adoption of this guidance to have any impact on the current period financial statements.

In May 2014, the FASB issued ASU No. 2014-09, "Revenue from Contracts with Customers (Topic 606)". This ASU supersedes the revenue recognition requirements in Accounting Standards Codification Topic 605, "Revenue Recognition". Under the new guidance, a company is required to recognize revenue when it transfers goods or renders services to customers at an amount that it expects to be entitled to in exchange for these goods or services. The new standard allows for either a full retrospective with or without practical expedients or a retrospective with a cumulative catch upon adoption transition method. This guidance is effective for the fiscal years beginning after December 15, 2016, with early adoption not permitted. In August 2015, the FASB issued ASU No. 2015-14, "Deferral of the

Effective Date", which states that the mandatory effective date of this new revenue standard will be delayed by one year, with early adoption only permitted in fiscal year 2017. During the second quarter of 2016, the FASB issued three amendments to the new revenue standard to address some application questions: ASU No. 2016-10, "Identifying Performance Obligations and Licensing", ASU No. 2016-11, "Rescission of SEC Guidance Because of Accounting Standards Updates 2014-09", and ASU No. 2016-12, "Narrow-Scope Improvements and Practical Expedients". These three amendments will be effective upon adoption of Topic 606. As of September 30, 2016, the Company has not yet determined which adoption method it will utilize or the effect that the adoption of this guidance will have on its consolidated financial statements.

3. SIGNIFICANT AGREEMENTS

University of Western Australia

In April 2013, the Company and UWA entered into an agreement under which an existing exclusive license agreement between the Company and UWA was amended and restated (the "Amended and Restated UWA License Agreement"). The Amended and Restated UWA License Agreement grants the Company specific rights to the treatment of DMD by inducing the skipping of certain exons. EXONDYS 51 falls under the scope of the license agreement. Under the Amended and Restated UWA License Agreement, the

Company may be required to make payments of up to $6.0 million in aggregate to UWA based on the successful achievement of certain development and sales milestones relating to EXONDYS 51 and up to five additional product candidates. The Company may also be obligated to make payments to UWA of up to $20.0 million upon the achievement of certain sales milestones. Additionally, the Company may also be required to pay a low-single-digit percentage royalty on net sales of products covered by issued patents licensed from UWA during the term of the Amended and Restated UWA License Agreement. However, the Company has the option to purchase future royalties up-front. Under this option, prior to the First Amendment (defined below), the Company could be required to make a one-time royalty payment of $30.0 million to UWA.

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

For the three and nine months ended September 30, 2016, the Company recorded $0.3 million and $7.3 million, respectively, relating to the development milestone and up-front payments to UWA as research and development expense in the unaudited condensed consolidated statement of operations and comprehensive loss.

Additionally, corresponding to the FDA approval and the subsequent commercial sale of EXONDYS 51, as defined in the Amended and Restated UWA License Agreement, the Company recorded a $1.0 million milestone as an in-license right in the unaudited condensed consolidated balance sheets as of September 30, 2016.

4. FAIR VALUE MEASUREMENTS

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

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

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

	Fair Value Measurement as of September 30, 2016			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 36,612	$ 36,612	$ —	$ —

Certificates of deposit	11,343	11,343	—	—
Total assets	$ 47,955	$ 47,955	$ —	$ —

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

The Company’s assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds and certificates of deposit. Money market funds are publicly traded mutual funds and are presented as cash equivalents in the unaudited condensed consolidated balance sheets as of September 30, 2016.

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

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

5. CASH, CASH EQUIVALENTS AND SHORT-TERM INVESTMENTS

It is the Company's policy to mitigate credit risk in its financial assets by maintaining a well-diversified portfolio that limits the amount of exposure as to maturity and investment type. As of September 30, 2016, the Company did not hold any available-for-sale securities. The weighted average maturity of the Company's available-for-sale securities as of December 31, 2015 was approximately four months.

The following tables summarize the Company's cash, cash equivalents and short-term investments for each of the periods indicated; as of September 30, 2016, there were no short-term investments.

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

6. OTHER CURRENT ASSETS AND OTHER NON-CURRENT ASSETS

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

	As of September 30, 2016	As of December 31, 2015
	(in thousands)	
Manufacturing-related deposits	$16,783	$ 13,070
Prepaid expenses	3,663	3,109
Other	1,556	1,201
Total other current assets	$22,002	$ 17,380

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

	As of September 30, 2016	As of December 31, 2015
	(in thousands)	
Manufacturing-related deposits	$4,084	—
Prepaid clinical expenses	3,725	4,228
Other	242	242
Total other non-current assets	$8,051	$ 4,470

7. ACCRUED EXPENSES

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

	As of September 30, 2016	As of December 31, 2015
	(in thousands)	
Accrued clinical and preclinical costs	$9,094	$ 9,587
Accrued employee compensation costs	7,649	8,189
Accrued contract manufacturing costs	7,605	4,830
Accrued professional fees	2,280	4,258
Accrued research costs	1,746	629
Other	1,165	1,560
Total accrued expenses	$29,539	$ 29,053

8. EQUITY FINANCING

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

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

9. RESTRUCTURING

In March 2016, the Company announced a long-term plan ("Corvallis plan") to consolidate all of the Company's operations to Massachusetts and reduce its workforce by approximately 19% as part of a strategic plan to increase operational efficiency. During the remainder of the year, the Company plans to close its facility in Corvallis, Oregon, which primarily focused on early-stage research and research manufacturing. As part of the consolidation, research activities and some employees will transition to the Company's facilities in Andover and Cambridge, Massachusetts. The consolidation efforts are planned to occur in four waves - May, October, November and December of 2016, with an estimated completion date of December 30, 2016. The restructuring costs of the Corvallis plan consist of costs associated with its workforce reduction and facility consolidation. The workforce reduction costs primarily relate to employee severance and benefits. Facility consolidation costs are primarily associated with non-cancellable lease obligations as well as accelerated depreciation for certain assets whose expected useful lives are shortened due to the consolidation. The Company has not determined the financial impact related to the non-cancellable lease obligation for the Corvallis facility but is currently obligated to make $4.3 million of lease payments after the estimated completion date of the consolidation plan. The Company estimates restructuring expenses of $1.8 million related to accelerated depreciation and workforce reduction costs, the latter of which will be accrued as earned over the service period for each employee.

In August 2016, the Company implemented a restructuring plan in Cambridge, Massachusetts ("Cambridge plan") and reduced its workforce by approximately 6%. The restructuring costs associated with the Cambridge plan consist of costs associated with workforce reduction totaling $0.7 million. The Cambridge plan was completed as of October 31, 2016.

For the three and nine months ended September 30, 2016, the Company recognized $1.3 million and $2.4 million of restructuring expenses, respectively, $1.0 million and $2.1 million, respectively, of which related to workforce reduction.

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

	For the Three Months Ended September 30, 2016 (in thousands)			For the Nine Months Ended September 30, 2016		
	Cash	Non-cash (1)	Total	Cash	Non-cash (2)	Total
Research and development	$628	$ 143	$771	$1,448	$ 336	$1,784
General and administration	367	126	493	471	168	639
Total restructuring expenses	$995	$ 269	$1,264	$1,919	$ 504	$2,423

(1) The non-cash restructuring expense relates to accelerated depreciation for certain assets.

(2) The non-cash restructuring expense relates to acceleration of stock option vesting and accelerated depreciation for certain assets.

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

	For the Three Months Ended September 30, 2016 (in thousands)	For the Nine Months Ended September 30, 2016
Restructuring reserve beginning balance	$371	$ —
Restructuring expenses incurred during the period	990	1,919
Adjustments to prior period estimates, net	5	—
Amounts paid during the period	(458)	(1,011)
Restructuring reserve ending balance	$908	$ 908

10. STOCK-BASED COMPENSATION

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

	For the Three Months Ended September 30,				For the Nine Months Ended September 30,			
	2016		2015		2016		2015	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options	1,050	$ 37.38	702,067	$ 24.05	1,214,426 (1)	$ 11.96	2,676,778	$ 14.67
Restricted stock awards	91,778 (2)	$ 48.94	65,000	$ 33.81	117,553 (3)	$ 41.22	181,783	$ 20.80

(1) Included in 2016 stock option grants are 287,500 options with performance conditions. As a result of the approval of EXONDYS 51, 25% of these performance grants vested immediately and another 25% were triggered to be eligible for vesting subject to the remaining service conditions of the awards. As of September 30, 2016, the performance conditions of the remaining 50% were not probable of being achieved. The remaining stock options granted during the periods presented in the table have only service-based criteria and vest over four years.

(2) The Company granted certain employees 91,778 restricted stock awards (“RSA”) with certain sales targets. If and when deemed probable that such performance milestones may be achieved within the required time frame, the Company may recognize up to $4.5 million of stock-based compensation related to these grants.

(3) Included in the 2016 RSA grants are 18,755 shares granted to certain employees in lieu of a portion of their 2015 annual bonus payments. These RSA grants were fully vested as of September 30, 2016. The remaining RSAs will be fully vested by June 2017.

Stock-based Compensation Expense

For the three months ended September 30, 2016 and 2015, total stock-based compensation expense was $9.6 million and $5.7 million, respectively. For the nine months ended September 30, 2016 and 2015, total stock-based compensation expense was $23.1 million and $25.8 million, respectively. As a result of the FDA approval of EXONDYS 51, certain performance criteria for options

with performance conditions were met during the quarter. The Company recognized approximately $3.7 million in stock-based compensation expense related to these options. Included in the amount for the nine months ended September 30, 2015 is $8.6 million of stock-based compensation expense incurred in connection with the resignation of the Company's former Chief Executive Officer. 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		For the Nine Months Ended	
	September 30,		September 30,	
	2016	2015	2016	2015
	(in thousands)			
Research and development	$2,674	$2,631	$7,527	$7,639
General and administrative	6,899	3,052	15,566	18,130
Total stock-based compensation expense	$9,573	$5,683	$23,093	$25,769

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		For the Nine Months Ended	
	September 30,		September 30,	
	2016	2015	2016	2015
	(in thousands)			
Stock options	$8,778	$4,801	$20,248	$23,451
Restricted stock awards	232	136	689	310
Stock appreciation rights	115	115	345	377
Employee stock purchase plan	448	631	1,811	1,631
Total stock-based compensation expense	$9,573	$5,683	$23,093	$25,769

11. NET LOSS PER SHARE

Basic net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding. Diluted net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding. Given that the Company generated a net loss for each of the periods presented, there is no difference between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive and, therefore, would be excluded from the diluted net loss per share calculation.

	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2016	2015	2016	2015
	(in thousands, except per share amounts)			
Net loss	$(56,742)	$(51,939)	$(178,813)	$(155,349)
Weighted-average number of shares of common stock and common stock equivalents outstanding:				
Weighted-average number of shares of common stock outstanding for computing basic loss per share	48,254	41,565	46,709	41,416
Dilutive effect of outstanding stock awards and stock options after application of the treasury stock method*	—	—	—	—
Weighted-average number of shares of common stock and dilutive common stock equivalents outstanding for computing diluted loss per share	48,254	41,565	46,709	41,416
Net loss per share — basic and diluted	$(1.18)	$(1.25)	$(3.83)	$(3.75)

*For the three and nine months ended September 30, 2016 and 2015, stock options, RSAs and stock appreciation rights to purchase approximately 6.3 million and 7.3 million shares of common stock, respectively, were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

12. 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. The Company records a liability in its consolidated financial statements for loss contingencies related to litigation when a loss is known or considered probable and the amount can be reasonably estimated. If the reasonable estimate of a known or probable loss is a range, and no amount within the range is a better estimate than any other, the minimum amount of the range is accrued. If a loss is reasonably possible but not known or probable, and can be reasonably estimated, the estimated loss or range of loss is disclosed.

On January 27, 2014 and January 29, 2014, purported class action complaints were filed in the U.S. District Court for the District of Massachusetts against the Company and certain of its current or former officers. The complaints were consolidated into a single action (Corban v. Sarepta, et al., No. 14-cv-10201) ("Corban") by order of the court on June 23, 2014, and plaintiffs were afforded 28 days to file a consolidated amended complaint. The plaintiffs' consolidated amended complaint, filed on July 21, 2014, sought to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 10, 2013 and November 11, 2013. The consolidated amended complaint alleged that Sarepta and certain of its current or former officers violated the federal securities laws in connection with disclosures related to eteplirsen and sought damages in an unspecified amount. On March 31, 2015, the Court granted Sarepta's motion to dismiss the plaintiffs' amended complaint. On August 12, 2015, the Court denied the plaintiffs' April 30, 2015 motion for leave seeking to file a further amended complaint, and on September 22, 2015, the Court dismissed the case. The plaintiffs filed a Notice of Appeal in the Court of Appeals for the First Circuit on September 29, 2015. On January 27, 2016, the plaintiffs filed a motion to vacate the District Court's order denying leave to amend and dismissing the case, during the pendency of which the plaintiffs' appeal was stayed. On April 21, 2016, the Court denied that motion. On May 19, 2016, the plaintiffs filed a motion to alter or amend the judgment. The Court denied that motion on May 20, 2016. A briefing schedule for the plaintiffs' appeal has been set by the First Circuit. An estimate of the possible loss or range of loss cannot be made at this time.

Another purported class action complaint was filed on December 3, 2014 in the U.S. District Court for the District of Massachusetts (Kader v. Sarepta et.al 1:14-cv-14318) ("Kader"), asserting that the Company and certain of its current or former officers violated Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5. The plaintiffs' amended complaint, filed on March 20, 2015, alleged that the 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. The plaintiffs sought compensatory damages and fees. On April 5, 2016, the Court granted Sarepta's motion to dismiss the amended complaint. On April 8, 2016, the plaintiffs filed a motion for leave to further amend the complaint, which Sarepta opposed on April 22, 2016. That motion remains pending. An estimate of the possible loss or range of loss cannot be made at this time.

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

On March 16, 2016 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., vs. Behrens et. al., 1:16-cv-10531). The claims allege that the defendants authorized the Company to make materially false and misleading statements about the Company's business prospects in connection with its development of eteplirsen from July 10, 2013 to the present. Plaintiffs seek unspecified damages, actions to reform and improve corporate governance and internal procedures, and attorneys' fees. The parties have agreed to stay the case pending resolution of the Corban and Kader cases. An estimate of the possible loss or range of loss cannot be made at this time.

Additionally, on September 23, 2014, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (Terry McDonald, derivatively on behalf of Sarepta Therapeutics, Inc., et. al vs. Goolsbee et. al., No. 10157). The claims allege, among other things, that (i) the Company's non-employee directors paid themselves excessive compensation fees for 2013, (ii) that the compensation for the Company's former CEO, Christopher Garabedian, was also excessive and such fees were the basis for Mr. Garabedian's not objecting to or stopping the excessive fees for the non-employee directors and (iii) that the disclosure in the 2013 proxy statement was deficient. The relief sought, among others, includes disgorgement and

rescindment of allegedly excessive or unfair payments and equity grants to Mr. Garabedian and the directors, unspecified damages plus interest, a declaration that the Company's Amended and Restated 2011 Equity Plan at the 2013 annual meeting was ineffective and a revote for approved amendments, correction of misleading disclosures and plaintiff's attorney fees. The parties have agreed to a Memorandum of Understanding concerning the settlement terms and do not believe that disposition of the McDonald suit will have a material financial impact on the Company. The parties are now engaged in the confirmatory discovery process that, when complete, will allow plaintiffs' counsel to represent to the court that the terms of the settlement are fair. Defendants have provided documents to plaintiffs, who are now in the process of reviewing the materials.

13. SUBSEQUENT EVENT

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

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

The Collaboration Agreement also grants the Company an option to expand the Licensed Territory. If the Company exercises this option, it will be liable for a one-time $10.0 million option fee as well as up to $7.0 million in regulatory milestone payments. For each licensed product, the Company may be liable for up to $82.5 million in sales milestone payments.

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

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

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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, 2015 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," "esti "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:

- the timing, investment and associated activities, including negotiating and entering into any additional commercial and supply contracts, scaling up manufacturing and hiring any additional personnel in connection with commercialization of EXONDYS 51 in the U.S.;

- Our expectations regarding the market size for EXONDYS 51 and our ability to manufacture sufficient amounts of EXONDYS 51 to meet actual commercial demand;

- our ability to verify the clinical benefit of EXONDYS 51 through our confirmatory trial(s) to obtain full approval from the United States Food and Drug Administration's ("FDA") and/or other regulatory authorities;

- third-party payor reimbursement and establishing and maintaining the marketing and distribution support for EXONDYS 51;

- our expectations regarding the timing of research, development, pre-clinical and clinical trial results, data and analyses relating to the safety profile and potential clinical benefits of EXONDYS 51 and our product candidates, our phosphorodiamidate morpholino oligomer ("PMO") chemistries, our other PMO-based chemistries and our other RNA-targeted technologies;

- Our ability to submit a Marketing Authorization Application ("MAA") for EXONDYS 51in the E.U. by end of year and obtain an approval from the European Medicines Agency ("EMA");

- our expectations regarding the FDA's interpretation of our data and information on our product candidates, PMO and PMO-based chemistries and RNA-targeted technologies and the impact on our business of the FDA's interpretations on our FDA submissions (including our investigational new drug ("IND") and new drug application ("NDA")), filing decisions by the FDA, advisory committee recommendations, and FDA product approval decisions and related timelines;

- our ability to respond to FDA requests during the regulatory process for each of our product candidates;

- our estimates regarding how long our currently available cash and cash equivalents will be sufficient to finance our operations and business plans and statements about our future capital needs;

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

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

- the potential safety, efficacy, potency and utility of our product candidates, chemistries and technologies in the treatment of Duchenne muscular dystrophy ("DMD") and other diseases;

- our expectations regarding the timing, completion and receipt of results from our ongoing development programs for our pipeline of product candidates including their potential consistency with prior results;

- our ability to effectively manage and execute post-marketing approval requirements for EXONDYS 51 and the clinical trial process for our product candidate, including our ability to successfully conduct our placebo-controlled study, ESSENCE using exon 45- and exon 53-skipping product candidates;

- our expectations regarding our ability to engage a number of manufacturers with sufficient capability and capacity to meet our manufacturing needs, including with respect to the manufacture of subunits, drug substance APIs and drug product, within the time frames and quantities needed to provide our product candidates to patients in larger scale clinical trials or in commercial quantities, and meet regulatory and Company quality control requirements;

- the impact of regulations as well as regulatory decisions by the FDA and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations;

- our expectations regarding the potential markets for our product candidates;

- our expectations regarding manufacturing and scale-up techniques to support the commercialization of EXONDYS 51;

- our expectations regarding our manufacturing and scale-up techniques and our ability to synthesize and purify our product candidates to adequately support clinical development and their potential commercialization;

- the potential acceptance of EXONDYS 51 and our product candidates, when introduced, in the marketplace;

- the possible impact of any competing products on the commercial success of EXONDYS 51 and our product candidates and our ability to compete against such products;

- the impact of potential difficulties in product development, manufacturing, or the commercialization of EXONDYS 51 and our product candidates, including difficulties in establishing and maintaining an appropriate commercial infrastructure necessary for the successful commercialization of EXONDYS 51;

- our expectations regarding the partnering opportunities and other strategic transactions that the Company has entered into or considers entering into in the future;

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

- our plans and ability to file and progress to issue additional patent applications to enhance and protect our new and existing technologies and programs;

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

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

- the impact of any consequences of the interference decisions including the final refusal of BioMarin claims in the exon 51 and exon 53 composition of matter interferences and the narrow claim BioMarin was allowed to pursue as a result of the exon 53 inference decision;

- the potential impact if the USPTO, other agencies or courts make a decision against us that could negatively impact the EXONDYS 51 commercialization such as a decision in the pending appeal of Interference No. 106,013 which could result in an infringement claim against us if the patents subject to the appeal are ultimately granted;

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

- our ability to enter into contracts, including collaborations or licensing agreements, with respect to our technology and product candidates, with third parties, including government entities;

- our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;

- the timing and outcomes of ongoing interference proceedings and related appeals;
- the impact of any litigation on us, including actions brought by stockholders;
- our ability to attract and retain key employees needed to execute our business plans and strategies and our expectations regarding our ability to manage the impact of any loss of key employees;
- our ability to comply with applicable environmental laws and regulations;
- our expectations relating to potential funding from government and other sources for the development of some of our product candidates;
- 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 the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. Applying our proprietary, highly-differentiated and innovative platform technologies, we are able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action. We are primarily focused on rapidly advancing the development of our potentially disease-modifying pipeline of DMD drug candidates. On September 19, 2016, the FDA granted accelerated approval for EXONDYS 51, indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 is studied in clinical trials under the name of eteplirsen and is marketed in the U.S. under the trademarked name of EXONDYS 51™ (eteplirsen) Injection.

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

regulatory agency or authority outside of the U.S.

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

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

(ii) EXONDYS 51 studies we are planning to initiate to comply with U.S. and/ or E.U. regulatory requirements for NDAs and marketing authorization applications, respectively (e.g. an IV study on participants between the ages of six months and four years in connection with our Pediatric Investigation Plan in the E.U., and two additional phase I studies);

(iii) studies we are planning to fulfill our post-marketing FDA requirements for EXONDYS 51 including a 2-year randomized double-blind, controlled trial in patients who have a confirmed mutation of the DMD gene amenable to exon 51 skipping and a 2-year controlled study in patients with confirmed mutations amenable to exon 45 or exon 53 that includes two well separated dose levels for each of the exon 45 skipping product candidate and the exon 53 skipping product candidate;

(iv) a dose-ranging study that we completed for our product candidate designed to skip exon 45 that has transitioned into an open-label study;

(v) a two-part randomized, double-blind, placebo-controlled, dose titration safety, tolerability and pharmacokinetics study for a product candidate designed to skip exon 53 for which we have completed Part I and have now transitioned into Part II, an open label efficacy and safety study; and

(vi) ESSENCE, a placebo-controlled study with product candidates designed to skip exons 45 and 53 which has begun enrolling patients in the U.S. and for which we plan to have sites in the E.U. and Canada.

In addition to our DMD program, we also have leveraged the capabilities of our RNA-targeted technology platforms to develop therapeutic candidates for the treatment of infectious diseases such as influenza, Marburg and Ebola under prior contracts with the U.S. Department of Defense ("DoD"); however, further development of these product candidates would be conditioned, in part, on obtaining additional funding, collaborations or emergency use. Our discovery and research programs include collaborations with various third parties and focus on developing therapeutics in rare, genetic, anti-bacterial, neuromuscular and central nervous system diseases. We are exploring the application of our PMO platform technology in various diseases.

We believe we have developed proprietary state-of-the-art manufacturing and scale-up techniques that allow synthesis and purification of EXONDYS 51 for commercial use and of our product candidates to support clinical development as well as commercialization. We have entered into certain manufacturing and supply arrangements with third-party suppliers which will in part utilize these techniques to support commercial production of EXONDYS 51 and production of certain of our product candidates and their components. We currently do not have any of our own internal mid-to-large scale manufacturing capabilities to support EXONDYS 51 or our product candidates.

The basis of our novel RNA-targeted therapeutics is the PMO. Our next generation PMO-based chemistries include PMO-X®, PMOplus® and PPMO. PMO and 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 and PMO-based compounds have demonstrated inhibition of messenger RNA ("mRNA") translation and alteration of pre-mRNA splicing. PMO and 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 and 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.

We have not generated any revenue from product sales through September 30, 2016, and we may never generate substantial revenue from product sales from the commercialization of EXONDYS 51 or our pipeline of product candidates. Even if we achieve substantial revenue from product sales, we are likely to continue to incur operating losses in the near term.

As of September 30, 2016, we had approximately $406.6 million of cash, cash equivalents and restricted cash and investments, consisting of $395.1 million of cash and cash equivalents and $11.5 million of restricted cash and investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve months. As of December 31, 2014, we had completed all development activities under our agreements with the DoD. We are currently open to possibilities for funding, collaboration and other avenues to support further development of these Ebola, Marburg and influenza product candidates. Without funding from the U.S. government, we likely will limit infectious disease research and development efforts, though we may pursue additional cash resources through public or private financings, seek additional government funding and establish collaborations with or license our technology to other companies.

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

or profitable operations from the commercialization of EXONDYS 51 or any of our product candidates.

Key Financial Metrics

Revenues

Product revenue. We recognize product revenue when there is persuasive evidence of an arrangement, delivery has occurred, price to the customer is fixed or determinable and collectability is reasonably assured. Revenue from product sales will be recognized when title and risk of loss have passed to the customer. Product revenue will be recorded net of applicable reserves for discounts and allowances.

Revenue from Research Contracts and Other Grants. We recognize revenue from research contracts and other grants during the period in which the related expenses are incurred and present such revenue and related expenses on a gross basis in the unaudited

condensed consolidated financial statements. Government contracts are subject to government audits, which may result in catch-up adjustments.

If a technology, right, product or service is separate from and independent of our performance under other elements of an arrangement, we defer recognition of non-refundable up-front fees if we have continuing performance obligations when the technology, right, product or service conveyed in conjunction with the non-refundable fee has no utility to the licensee. In addition, if we have continuing involvement through research and development services that are required because of our know-how or because the services can only be performed by us, such up-front fees are deferred and recognized over the period of continuing involvement. As of September 30, 2016, we had deferred revenue of $3.3 million, which represents up-front fees which we may recognize as revenue upon settlement of certain obligations.

Expenses

Research and Development. Research and development expenses consist of costs associated with research activities as well as costs associated with our product development efforts, conducting preclinical studies, clinical trials and manufacturing activities.

Direct research and development expenses associated with our programs include clinical trial site costs, clinical manufacturing costs, costs incurred for consultants 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 and product candidates, enter or proceed through additional clinical development. We are currently conducting various clinical trials for EXONDYS 51, including a confirmatory trial in the U.S. We completed Part I and are conducting Part II of a Phase I/IIa clinical trial for an exon 53 skipping product candidate in the E.U. We have also initiated a dose-ranging study for our exon 45 skipping product candidate in the U.S. We have begun enrolling in the U.S. and are also planning to initiate enrollment in the E.U. for a placebo-controlled confirmatory study with product candidates designed to skip exons 45 and 53. The remainder of our research and development programs are in various stages of research and preclinical development. However, our current research and development efforts may not result in additional 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 the other 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 substantial revenue from the commercialization of EXONDYS 51 or any of our other product candidates. The time frame for development of any product candidate, associated development costs and the probability of regulatory and commercial success vary widely.

General and Administrative. General and administrative expenses consist principally of salaries, benefits, stock-based compensation and related costs for personnel in our executive, finance, legal, information technology, business development, and human resource, 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.

Interest (Expense) Income and Other, Net. Interest (expense) income and other, net, primarily consists of interest expense, interest income on our cash, cash equivalents and restricted investments and rental income. Our cash

equivalents and investments consist of money market investments and certificates of deposit. Interest expense includes interest incurred on our senior secured term loan and our mortgage loans related to our Corvallis, Oregon property, a substantial portion of which has been leased to a third party since November 2011. Rental income is from leasing excess space in some of our facilities.

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, revenue 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. For any given individual estimate or assumption we make, there may also be other estimates or assumptions that are reasonable. 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 when 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.

The policies that we believe are the most critical to aid the understanding of our financial results include:

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

There have been no material 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, 2015.

Results of Operations for the Three and Nine Months Ended September 30, 2016 and 2015

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

	For the Three Months Ended			
	September 30, 2016	2015	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues	$—	$—	$—	NA
Operating expenses:				
Research and development	34,349	36,673	(2,324)	(6)%
General and administrative	22,184	15,090	7,094	47 %
Total operating expenses	56,533	51,763	4,770	9 %
Operating loss	(56,533)	(51,763)	(4,770)	9 %
Other loss:				
Interest expense and other, net	(209)	(176)	(33)	19 %
Net loss	$(56,742)	$(51,939)	$(4,803)	9 %
Net loss per share — basic and diluted	$(1.18)	$(1.25)	$0.07	(6)%

	For the Nine Months Ended			
	September 30, 2016	2015	Change	Change
	(in thousands, except per		$	%

	share amounts)				
Revenues	$—	$—	$—	NA	
Operating expenses:					
Research and development	117,523	105,018	12,505	12	%
General and administrative	60,812	50,714	10,098	20	%
Total operating expenses	178,335	155,732	22,603	15	%
Operating loss	(178,335)	(155,732)	(22,603)	15	%
Other income (loss):					
Interest (expense) income and other, net	(478)	383	(861)	(225)	%
Net loss	$(178,813)	$(155,349)	$(23,464)	15	%
Net loss per share — basic and diluted	$(3.83)	$(3.75)	$(0.08)	2	%

Revenues

As of December 31, 2014, we had completed all development activities related to our contracts with the U.S. government. Therefore, no revenue was recognized for the three and nine months ended September 30, 2016 or 2015 in connection with such contracts. The majority of the revenue under our U.S. government contracts has been recognized as of September 30, 2016 and only revenue for contract finalization, if any, is expected in the future.

We have not generated any revenue from product sales through September 30, 2016, and we may never generate substantial revenue from product sales from the commercialization of EXONDYS 51 or our pipeline of product candidates.

Research and Development Expenses

Our research and development expenses represent a substantial percentage of our total operating expenses, which primarily consist of costs associated with research activities as well as costs associated with our product development efforts, conducting preclinical studies, clinical trials and manufacturing activities. 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, including salaries, stock-based compensation and allocation of our facility costs, are not tracked by project, as the costs may benefit multiple projects. The following tables summarize research and development expenses by project for each of the periods indicated:

	For the Three Months Ended September 30,			
	2016	2015	Change $	Change %
	(in thousands)			
EXONDYS 51	$17,966	$18,853	$(887)	(5)%
Exon 53	2,544	1,276	1,268	99 %
Exon 45	1,837	1,181	656	56 %
Other projects	157	173	(16)	(9)%
Internal research and development expenses	11,845	15,190	(3,345)	(22)%
Total research and development expenses	$34,349	$36,673	$(2,324)	(6)%

	For the Nine Months Ended September 30,			
	2016	2015	Change $	Change %
	(in thousands)			
EXONDYS 51	$64,337	$52,353	$11,984	23 %
Exon 53	7,584	3,446	4,138	120 %
Exon 45	4,302	5,130	(828)	(16)%
Other projects	1,222	1,218	4	0 %
Internal research and development expenses	40,078	42,871	(2,793)	(7)%
Total research and development expenses	$117,523	$105,018	$12,505	12 %

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

	For the Three Months Ended September 30,			
	2016	2015	Change $	Change %
	(in thousands)			
Clinical and manufacturing expenses	$20,773	$20,801	$(28)	(0)%
Compensation and other personnel expenses	5,477	6,351	(874)	(14)%
Stock-based compensation	2,674	2,631	43	2 %
Facility-related expenses	1,645	2,395	(750)	(31)%
Professional services	1,718	2,067	(349)	(17)%
Preclinical expenses	612	731	(119)	(16)%
Research and other	1,450	1,697	(247)	(15)%
Total research and development expenses	$34,349	$36,673	$(2,324)	(6)%

	For the Nine Months Ended September 30,			
	2016	2015	Change $	Change %
	(in thousands)			
Clinical and manufacturing expenses	$65,681	$57,477	$8,204	14 %
Compensation and other personnel expenses	18,116	18,488	(372)	(2)%
Up-front license payment to UWA	7,000	—	7,000	NA
Stock-based compensation	7,527	7,639	(112)	(1)%
Facility-related expenses	5,736	7,303	(1,567)	(21)%
Professional services	5,757	5,888	(131)	(2)%
Preclinical expenses	2,583	3,239	(656)	(20)%
Research and other	5,123	4,984	139	3 %
Total research and development expenses	$117,523	$105,018	$12,505	12 %

Research and development expenses for the three months ended September 30, 2016 decreased by $2.3 million, or 6%, compared with the three months ended September 30, 2015. The decrease was primarily due to decreases of $0.9 million in compensation and other personnel expenses and $0.8 million in facility-related expenses.

Research and development expenses for the nine months ended September 30, 2016 increased by $12.5 million, or 12%, compared with the nine months ended September 30, 2015. The increase was primarily due to an increase of $8.2 million in clinical and manufacturing expenses driven by increased enrollment in our ongoing clinical trials and a $7.0 million up-front license payment to the University of Western Australia (“UWA”) partially offset by a decrease of $1.6 million in facility-related expenses.

General and Administrative Expenses

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

	For the Three Months Ended September 30,			
	2016	2015	Change $	Change %
	(in thousands)			
Compensation and other personnel expenses	$8,238	$4,160	$4,078	98 %
Stock-based compensation	6,899	2,897	4,002	138 %
Professional services	4,283	6,223	(1,940)	(31)%
Facility-related expenses	1,291	815	476	58 %
Other	1,473	995	478	48 %
Total general and administrative expenses	$22,184	$15,090	$7,094	47 %

	For the Nine Months Ended September 30,

	2016	2015	Change $	Change %
	(in thousands)			
Compensation and other personnel expenses	$22,947	$11,289	$11,658	103 %
Stock-based compensation	15,566	9,417	6,149	65 %
Professional services	13,894	14,477	(583)	(4)%
Estimated severance expenses	—	9,182	(9,182)	(100)%
Facility-related expenses	3,385	2,624	761	29 %
Other	5,020	3,725	1,295	35 %
Total general and administrative expenses	$60,812	$50,714	$10,098	20 %

General and administrative expenses for the three months ended September 30, 2016 increased by $7.1 million, or 47%, compared with the three months ended September 30, 2015. This was primarily due to increases of $4.1 million in compensation and other personnel expenses primarily driven by an increase in commercial headcount, $4.0 million in stock-based compensation primarily due to the FDA approval of EXONDYS 51 which triggered certain performance grants to become eligible for vesting, and $0.5 million in facility-related expenses. The increase was partially offset by a decrease of $1.9 million in professional services primarily due to the ramp-down of certain external commercial activities to assist in the product launch of EXONDYS 51.

General and administrative expenses for the nine months ended September 30, 2016 increased by $10.1 million, or 20%, compared with the nine months ended September 30, 2015. This was primarily due to increases of $11.7 million in compensation and other personnel expenses primarily driven by an increase in commercial headcount, $6.1 million in stock-based compensation primarily driven by an increase in headcount as well as the FDA approval of EXONDYS 51 which triggered certain performance grants to become eligible for vesting, and $0.8 million in facility-related expenses. The increase was partially offset by a decrease in severance expense of $9.2 million as a result of the resignation of our former CEO in March 2015.

Interest (Expense) Income and Other, Net

For the three months ended September 30, 2016, interest expense and other, net was relatively flat compared with the three months ended September 30, 2015.

For the nine months ended September 30, 2016, interest expense and other, net was approximately $0.5 million. For the nine months ended September 30, 2015, interest income and other, net was approximately $0.4 million. The unfavorable change was primarily driven by interest expense incurred in connection with the $20.0 million senior secured term loan.

Liquidity and Capital Resources

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

	As of September 30, 2016	As of December 31, 2015	Change $	Change %
	(in thousands)			
Financial assets:				
Cash and cash equivalents	$395,140	$80,304	$314,836	392 %
Short-term investments	—	112,187	(112,187)	(100)%
Restricted cash and investments	11,479	11,478	1	0 %
Total cash, cash equivalents and investments	$406,619	$203,969	$202,650	99 %
Borrowings:				
Long-term debt	$18,598	$20,905	$(2,307)	(11)%
Notes payable	—	2,493	(2,493)	(100)%
Total borrowings	$18,598	$23,398	$(4,800)	(21)%
Working capital				
Current assets	$434,744	$224,543	$210,201	94 %
Current liabilities	64,933	62,294	2,639	4 %
Total working capital	$369,811	$162,249	$207,562	128 %

Our principal sources of liquidity are from both equity and debt financings. Our principal uses of cash are research and development expenses, general and administrative expenses, capital expenditures and other working capital requirements.

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

- the timing and costs associated with commercialization of EXONDYS 51;
- the timing and costs of building out our manufacturing capabilities;
- the timing of advanced payments related to our future inventory commitments;
- the timing and costs associated with our clinical trials and preclinical studies; 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 our business development efforts, and we may seek additional financing primarily from, but not limited to, the sale and issuance of equity, debt securities or the licensing or sale of our technology. Financing may not be available when and as needed or, if available, financings may not be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we

require, our business and results of operations will be negatively impacted. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

Cash Flows

	For the Nine Months Ended September 30,			
	2016	2015	Change $	Change %
	(in thousands)			
Cash provided by (used in)				
Operating activities	$(164,587)	$(118,193)	$(46,394)	39 %
Investing activities	108,581	78,229	30,352	39 %
Financing activities	370,842	22,232	348,610	1,568 %
Increase (decrease) in cash and cash equivalents	$314,836	$(17,732)	$332,568	(1,876)%

Operating Activities. The increase in cash used in operating activities of $46.4 million for the nine months ended September 30, 2016 compared with the nine months ended September 30, 2015 was primarily due to an increase of $23.5 million in net loss primarily driven by increases in research and development and general and administrative expenses, a decrease of $2.6 million in non-cash adjustments and $20.4 million of unfavorable changes in operating assets and liabilities.

Investing Activities. The cash provided by investing activities increased by $30.4 million for the nine months ended September 30, 2016 compared with the nine months ended September 30, 2015. This was primarily driven by the purchases of $49.6 million in available-for-sale securities and $10.7 million in restricted investments during the nine months ended September 30, 2015 compared to no purchases of available-for-sale securities or restricted investments for the comparable period in 2016. This was partially offset by a decrease of $29.8 million from the sale and maturity of available-for-sale securities.

Financing Activities. The increase in cash provided by financing activities of $348.6 million for the nine months ended September 30, 2016 compared with the nine months ended September 30, 2015 was primarily driven by $365.0 million in proceeds from the issuance of approximately 5.8 million shares and 2.1 million shares of common stock at an offering price of $59.75 and $17.84 per share in September 2016 and June 2016, respectively, and an increase of $5.8 million from option exercises and Employee Stock Purchase Program purchases. This was partially offset by $5.1 million in repayment of debt in connection with the promissory note related to the May 2014 acquisition of our Andover, Massachusetts facility and the senior secured term loan taken out in June 2015.

Milestone Obligations

As of September 30, 2016, we were obligated to make up to $91.5 million of future development, up-front royalty and sales milestone payments associated with certain of our collaboration and license agreements. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory or sales milestones. For the three and nine months ended September 30, 2016, we recorded $0.3 million and $7.3 million, respectively, relating to development milestone and up-front payments to UWA in connection with the license agreement and its first amendment as research and development expense in the unaudited condensed consolidated statement of operations and comprehensive loss. Additionally, corresponding to the FDA approval and the subsequent commercial sale of EXONDYS 51, as defined in the Amended and Restated UWA License Agreement, we recorded a $1.0 million sales milestone payment to UWA as an intangible asset in the unaudited condensed consolidated balance

sheets as of September 30, 2016. Because the achievement of all other milestones had not occurred as of September 30, 2016, such contingencies have not been recorded in our financial statements.

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 September 30, 2016.

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 September 30, 2016, we had approximately $406.6 million of cash, cash equivalents and restricted investments, comprised of $395.1 million of cash and cash equivalents and $11.5 million of restricted cash and investments. As of September 30, 2016, our cash equivalents consist of money market investments whose fair value is not subject to change as a result of potential changes in market interest rates and we don't have other investments that are sensitive to change in market interest rate.

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 September 30, 2016, 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 CEO and our CFO, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of September 30, 2016, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarterly period ended September 30, 2016, there were no changes in the Company's internal controls over financial reporting that have materially affected or are reasonably likely to materially affect the Company's internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings

For material legal proceedings, please read Note 12, 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, including the Annual Report on Form 10-K for the year ended December 31, 2015, are descriptions of risks and uncertainties that could cause actual results to differ materially from the results contemplated by the forward-looking statements contained in this report. Because of the following factors, as well as other variables affecting our operating results, past financial performance should not be considered a reliable indicator of future performance and investors should not use historical trends to anticipate results or trends in future periods. The risks and uncertainties described below are not the only ones facing us. Other events that we do not currently anticipate or that we currently deem immaterial also affect our results of operations and financial condition.

Risks Related to Our Business

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

On September 19, 2016, the FDA granted accelerated approval for EXONDYS 51 as a therapeutic treatment for DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 is commercially available. The commercial success of EXONDYS 51 will depend on a number of factors, including:

- the effectiveness of our sales, managed markets and marketing efforts;

- FDA-mandated package insert requirements and the time it would take us to comply with any related FDA post-marketing requirements;

- demonstration and/or confirmation of clinical efficacy and safety and acceptance of the same by the medical community;

- the occurrence of any side effects, adverse reactions or misuse, or any unfavorable publicity in these areas;

- whether EXONDYS 51 can consistently be manufactured in commercial quantities and at acceptable costs;

- the cost-effectiveness of the product;

- adequate reimbursement by third parties, including government payers, managed care organizations and private health insurers;

- our ability to comply with the FDA requirements, and achieve the required clinical endpoints in the studies included in the EXONDYS 51 approval letter including our ability to successfully conduct and achieve the endpoints in the two-year post-approval study required by the FDA to verify EXONDYS 51's clinical benefit;

- the need for, and success of, other confirmatory trials and post-marketing requirements;

- the development or commercialization of competing products or therapies for the treatment of DMD, or its symptoms;

- marketing and distribution support for EXONDYS 51;

- our ability to remain compliant with laws and regulations that apply to us and our commercial activities;

- the actual market-size for EXONDYS 51, which may be different than expected;

- the sufficiency of our drug supply to meet commercial and clinical demands which could be negatively impacted if our projections on the potential number of amenable patients and their average weight are inaccurate, we are subject to unanticipated regulatory requirements that increase our drug supply needs, our current drug supply is destroyed or negatively impacted at our manufacturing sites, storage sites or in transit, or it takes longer than we project for the number of patients we anticipate to get on EXONDYS 51 and any significant portion of our EXONDYS 51 supply expires before we are able to sell it; and

-

our ability to obtain regulatory approvals to commercialize EXONDYS 51 in markets outside of the U.S.

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

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

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

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

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

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

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

If these third parties were to cease providing quality manufacturing and related services to us, and we are not able to engage appropriate replacements in a timely manner, our ability to manufacture EXONDYS 51 or our product candidates in sufficient quality and quantity required for commercial use of EXONDYS 51 and/ or for planned pre-clinical testing, clinical trials and potential commercial use of our product candidates would be adversely affected.

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

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

Our contract manufacturers are required to produce our materials, APIs and drug products under current Good Manufacturing Practice regulations ("cGMP"). We and our contract manufacturers are subject to periodic inspections by

the FDA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations. We do not have control over a third-party manufacturer's compliance with these regulations and requirements. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of EXONDYS 51 and our product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively. The failure to achieve and maintain compliance with cGMP and other applicable government regulations, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in product recalls, patient injury or death. If our contract manufacturers fail to adhere to applicable cGMP and other applicable government regulations, or experience manufacturing problems, we will suffer significant consequences, including product seizures or recalls, postponement or cancellation of clinical trials, loss or delay of product approval, fines and sanctions, loss of revenue, termination of the development of a product candidate, reputational damage, shipment delays, inventory shortages, inventory write-offs and other product-related charges and increased manufacturing costs. If we experience any of these results, we may not be able to successfully commercialize EXONDYS 51.

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

We are working to increase manufacturing capacity and scale up production of some of the components of our drug products. During the remainder of 2016, our focus remains on (i) achieving larger-scale manufacturing capacity for EXONDYS 51 throughout the manufacturing supply chain and (ii) continuing to increase material and API production capacity to provide the anticipated amounts of drug product needed for our planned studies for our product candidates. We may not be able to successfully increase manufacturing capacity or scale up the production of materials, APIs and drug products, whether in collaboration with third-party manufacturers or on our own, in a manner that is safe, compliant with cGMP conditions or other applicable legal or regulatory requirements, in a cost-effective manner, in a time frame required to meet our timeline for commercialization, clinical trials and other business plans, or at all. Compliance with cGMP requirements and other quality issues may arise during our efforts to increase manufacturing capacity and scale up production with our current or any new contract manufacturers. These issues may arise in connection with the underlying materials, the inherent properties of EXONDYS 51 or a product candidate, EXONDYS 51 or a product candidate in combination with other components added during the manufacturing and packaging process or during shipping and storage of the APIs or finished drug product. In addition, in order to release EXONDYS 51 for commercial use and demonstrate stability of product candidates for use in late stage clinical trials (and any subsequent drug products for commercial use), our manufacturing processes and analytical methods must be validated in accordance with regulatory guidelines. We may not be able to successfully validate, or maintain validation of, our manufacturing processes and analytical methods or demonstrate adequate purity, stability or comparability of EXONDYS 51 or our product candidates in a timely or cost-effective manner, or at all. If we are unable to successfully validate our manufacturing processes and analytical methods or to demonstrate adequate purity, stability or comparability, the commercial availability of EXONDYS 51 and the continued development and/or regulatory approval of our product candidates may be delayed, which could significantly harm our business.

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

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

We will rely on third parties to commercially distribute EXONDYS 51 to patients. We have contracted with a third-party logistics company to warehouse EXONDYS 51 and with specialty pharmacies to sell and distribute it to patients. A specialty pharmacy is a pharmacy that specializes in the dispensing of medications for complex or chronic conditions that require a high level of patient education and ongoing management. We are also planning to contract with a third-party call center to help us with some or all of the following: coordinate prescription intake and distribution, reimbursement adjudication, patient financial support, and ongoing compliance support. This distribution network will require significant coordination with our sales and marketing and finance organizations. In addition, failure to coordinate financial systems could negatively impact our ability to accurately report product revenue from EXONDYS 51. If we are unable to effectively manage the distribution process, the commercial launch and sales of EXONDYS 51, as well as any future products we may commercialize, could be delayed or severely compromised and our results of operations may be harmed.

In addition, the use of specialty pharmacies and a call center involves certain risks, including, but not limited to, risks that these organizations will:

- not provide us with accurate or timely information regarding their inventories, the number of patients who are using EXONDYS 51 or serious adverse events and/or product complaints regarding EXONDYS 51;

- not effectively sell or support EXONDYS 51;

- reduce or discontinue their efforts to sell or support EXONDYS 51;

- not devote the resources necessary to sell EXONDYS 51 in the volumes and within the time frame we expect;

- be unable to satisfy financial obligations to us or others; or

- cease operations.

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

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

We have hired a commercial team and put in the organizational infrastructure we believe we need for a successful commercial launch of EXONDYS 51. We will need to commit significant time and financial and managerial resources to maintain and further develop our marketing and sales force to ensure they have the technical expertise required to address any challenges we may face with the commercialization of EXONDYS 51. Factors that may inhibit our efforts to maintain and develop our commercialization capabilities include:

- an inability to retain an adequate number of effective commercial personnel;
- an inability to train sales personnel, who may have limited experience with our company or EXONDYS 51, to deliver a consistent message regarding EXONDYS 51 and be effective in convincing physicians to prescribe EXONDYS 51;
- an inability to equip sales personnel with effective materials, including medical and sales literature to help them educate physicians and our healthcare providers regarding EXONDYS 51 and its proper administration;
- unforeseen costs and expenses associated with maintaining and further developing an independent sales and marketing organization.

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

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

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

Under the accelerated approval provisions, the FDA is requiring that the Company complete various post-approval requirements including conducting a clinical trial to verify the drug’s clinical benefit. If the trial fails to verify clinical benefit, the FDA may initiate proceedings to withdraw approval of the drug. These post-approval requirements could impose significant burdens and costs on us. Failure to meet post-approval commitments, including obtaining positive safety and efficacy data from our confirmatory studies for EXONDYS 51, would lead to negative regulatory action from the FDA, which could include withdrawal of regulatory approval of EXONDYS 51.

Manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP regulations. If we or a regulatory agency discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with a facility where the product is manufactured, a regulatory agency may impose restrictions on that product or the manufacturer, including requiring implementation of a risk evaluation and mitigation strategy program, withdrawal of the product from the market or suspension of manufacturing. If we or the manufacturing facilities for

EXONDYS 51 fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to applications submitted by us;
- impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products or require us to initiate a product recall.

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

In the future, we may seek to commercialize EXONDYS 51 in foreign countries outside of the U.S. In order to market any products outside of the U.S., we must comply with numerous and varying regulatory requirements of other jurisdictions regarding safety and efficacy. Approval procedures vary among jurisdictions and can involve product testing and administrative review periods different from, and greater than, those in the U.S.

Regulatory approval in one jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory processes in others. Failure to obtain regulatory approvals in other jurisdictions or any delay or setback in obtaining such approvals could adversely affect our business and financial condition.

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

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

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

- the effectiveness of our sales and marketing organizations and distribution networks;

- the ability of patients or providers to be adequately reimbursed for EXONDYS 51 in a timely manner from government and private payors; and

- 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.

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 3,500-5,000 males born worldwide, of which up to 13% are estimated to be amenable to exon-51 skipping. Our estimate of the size of the patient population is based on published studies as well as internal analyses. If the results of these studies or our analysis of them do not accurately reflect the number of patients with DMD, our assessment of the market may be inaccurate, making it difficult or impossible for us to meet our revenue goals, or to obtain and maintain profitability. Since EXONDYS 51 targets a small patient population, the per-patient drug pricing must be high in order to recover our development and manufacturing costs, fund adequate patient support programs, fund additional research and achieve profitability. We may be unable to maintain or obtain sufficient sales volumes at a price high enough to justify our product development efforts and our sales, marketing and manufacturing expenses.

We have been granted orphan drug designations in the U.S. and in the E.U. for certain of our product candidates, however, there can be no guarantee that we will maintain orphan status for these product candidates nor that we will receive orphan drug approval and prevent third parties from developing and commercializing products that are competitive to these product candidates in the absence of other barriers to entry.

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

We also have been granted orphan medicinal product designations in the European Union ("E.U.") for two of our product candidates in DMD (including EXONDYS 51). Product candidates granted orphan status in Europe can be provided with up to ten years of marketing exclusivity, meaning that another application for marketing authorization of a later, similar medicinal product for the same therapeutic indication will generally not be approved in Europe during that time period. Although we may have product candidates that obtain orphan drug exclusivity in Europe, the orphan status and associated exclusivity period may be modified for

several reasons, including a significant change to the orphan medicinal product designations or status criteria after-market authorization of the orphan product (e.g. , product profitability exceeds the criteria for orphan drug designation), problems with the production or supply of the orphan drug, or a competitor drug, although similar, is safer, more effective or otherwise clinically superior than the initial orphan drug.

As discussed above, we are not guaranteed to receive or maintain orphan status for our current or future product candidates, and if our product candidates that are granted orphan status were to lose their status as orphan drugs or the marketing exclusivity provided for them in the U.S. or the E.U., our business and results of operations could be materially adversely affected. While orphan status for any of our products, if granted or maintained, would provide market exclusivity in the U.S. and the E.U. for the time periods specified above, we would not be able to exclude other companies from manufacturing and/or selling products using the same active ingredient for the same indication beyond the exclusivity period applicable to our product on the basis of orphan drug status. In addition, we cannot guarantee that another company will not receive approval to market a product candidate that is granted orphan drug status in the U.S. or the E.U. for a product candidate that has the same active ingredient or is a similar medicinal product for the same indication as any of our product candidates for which we plan to file a new drug application ("NDA") or marketing authorization application ("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 E.U., as applicable. Furthermore, application of the orphan drug regulations in the U.S. and Europe is uncertain, and we cannot predict how the respective regulatory bodies will interpret and apply the regulations to our or our competitors' product candidates.

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

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

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

Our ability to successfully commercialize EXONDYS 51 in the U.S. will depend in part on the coverage and reimbursement levels set by governmental authorities, private health insurers and other third-party payors. Third-party payors are increasingly challenging the effectiveness of and prices charged for medical products and services. We may not obtain adequate third-party coverage or reimbursement for EXONDYS 51, or we may be required to sell

EXONDYS 51 at an unsatisfactory price.

We expect that private insurers will consider the efficacy, cost-effectiveness and safety of EXONDYS 51 in determining whether to approve reimbursement for EXONDYS 51 and at what level. Obtaining these approvals can be a time consuming and expensive process. Our business would be materially adversely affected if we do not receive approval for reimbursement of EXONDYS 51 from private insurers on a timely or satisfactory basis. Our business could also be adversely affected if private insurers, including managed care organizations, the Medicare or Medicaid programs or other reimbursing bodies or payors limit the indications for which EXONDYS 51 will be reimbursed.

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

country in which reimbursement is sought, limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed.

We expect to experience pricing pressures in connection with the sale of EXONDYS 51 and our future products due to the healthcare reforms discussed below, as well as the trend toward programs aimed at reducing healthcare costs, the increasing influence of health maintenance organizations and additional legislative proposals.

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

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

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

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

Other than EXONDYS 51 which the FDA approved for use in the U.S. in September 2016 and for which we plan to file an MAA by end of year with EMA, our most advanced product candidates are exon 45 and 53 skipping products. We are in the process of conducting, starting or planning various EXONDYS 51 clinical studies including studies that are required to comply with regulatory NDA and/or MAA filing requirements as well as studies we need to conduct to comply with our post-marketing FDA requirements to verify and describe clinical benefit. The exon 53-skipping product candidate, which we are working on with the SKIP-NMD consortium, is currently in the clinic in EU. The Part I dose-titration portion of this Phase I/IIa study has been completed and Part II open label portion of the study is ongoing. We have also completed the dose titration portion and are conducting the open-label portion of a study for our exon 45-skipping product candidate. Additionally, we are enrolling patients in the U.S. and working towards initiating sites in the E.U. and Canada for a clinical trial using exon 45- and 53-skipping product candidates, which we refer to as the ESSENCE study. The remainder of our product candidates are in discovery or early stages of development. These product candidates will require significant further development, financial resources and personnel to develop into commercially viable products and obtain regulatory approval, if at all. Currently, our exon 45-skipping product candidate, the exon 53-skipping product candidate we are developing with the SKIP-NMD consortium, each for DMD, and radavirsen (formerly AVI-7100) for influenza are in active clinical development. Our other product candidates, including our anti-bacterials and AVI-7537 in Ebola/ and AVI-7288, are in discovery, pre-clinical

development or inactive. Given the FDA approval of EXONDYS 51, we expect that much of our effort and many of our expenditures over the next several years will be devoted to clinical development and regulatory activities associated with EXONDYS 51 and other exon-skipping candidates as part of our larger pan-exon strategy in DMD, our infectious 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 be delayed in or may not be able to successfully submit an MAA to EMA that leads to an approval of EXONDYS 51 in the E.U.

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

Our RNA-targeted platform, utilizing proprietary PMO-based technology has only been incorporated into one therapeutic commercial product to date, EXONDYS 51, however, our confirmatory trials for EXONDYS 51 must verify and describe the clinical benefits in order for EXONDYS 51 to remain approved in the U.S. All of our product candidates to date use our PMO-based

technology. Although we have conducted and are in the process of conducting clinical studies with EXONDYS 51, an exon 45-skipping product candidate and an exon 53-skipping product candidate and pre-clinical studies with our other product candidates that use our PMO-based antisense technology, additional studies may be needed to determine the safety and efficacy of our PMO-based antisense technology. In addition, nonclinical models used to evaluate the activity and toxicity of product candidate compounds are not necessarily predictive of toxicity or efficacy of these compounds in the treatment of human disease. As such, there may be substantially different results observed in clinical trials from those observed in pre-clinical studies. Any failures or setbacks in developing or utilizing our PMO-based technology, 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.

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 would materially impair our ability to generate 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., cannot be assured, may be significantly delayed or may never be achieved for various reasons including the following:

- Our non-clinical, clinical, Chemistry, Manufacturing and Controls (“CMC”) and other data and analyses from past, current and future studies for any of our product candidates may not be sufficient to meet regulatory requirements for submissions of a marketing application or approvals. The FDA could disagree with our beliefs, interpretations and conclusions regarding data we provide in connection with an NDA submission for one of our product candidates, and may delay, reject or refuse to file or approve any NDA submission we make or identify additional requirements for product approval in a complete response letter to be submitted upon completion, if ever. In addition, an advisory committee could determine 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 deny approval of our product candidates based on their review of the data or other factors.

- 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 novel endpoints, such as natural history data and dystrophin measures, is uncertain due to, among other factors, evolving interpretations of a new therapeutic class, the broad discretion of regulatory authorities, lack of precedent, varying levels of applicable expertise of regulators or their advisory committees, scientific developments, changes in the competitor landscape, shifting political priorities and changes in applicable laws, rules or regulations and interpretations of the same. We cannot be sure that any of our product candidates will qualify for accelerated approval under Food and Drug Administration Safety and Innovation Act 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, we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned investigational new drug applications (“INDs”) and NDAs

for our product candidates, in a timely manner, or at all. Examples of such requests or requirements could include, but are not limited to, conducting additional or redesigned trials and procedures (e.g., additional patient muscle biopsies and dystrophin analyses), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, an 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 review of our NDA or result in a decision by the Company not to proceed with the development of a product candidate or an NDA submission for a product candidate based on feedback from regulators.

- We may not have the resources required to meet regulatory requirements and successfully navigate what is generally a lengthy, expensive and extensive approval process for commercialization of drug product candidates. Any failure on our part to respond to these requirements in a timely and satisfactory manner could significantly delay or negatively impact confirmatory study timelines and/or the development plans we have for the exon 53- and exon 45-skipping or other product candidates. Responding to requests from regulators and meeting requirements for clinical studies, submissions, filings, advisory committees and approvals may require substantial personnel, financial or other resources, which, as a small pre-commercial 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 and the timing of regulatory decisions with respect to any NDA submissions.

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

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

Even after approval and commercialization of a product candidate, we would 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.

Our pre-clinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, which could prevent or significantly delay their regulatory approval.

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive pre-clinical and clinical studies that the product candidate is safe and effective in humans. Ongoing and future pre-clinical and clinical trials of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. Furthermore, success in pre-clinical and early clinical trials does not ensure that the subsequent trials will be successful, nor does it predict final results of a confirmatory trial. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, 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. For example, we cannot provide assurances that data from any of our ongoing studies will be positive and consistent through the study periods or that the interpretation by regulators, such as the FDA, of the data we collect for our

product candidates will be consistent with our interpretations.

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, even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, certain federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. We will be subject to healthcare fraud and abuse and patient privacy regulation by both the federal government and the states in which we conduct 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 payers that are false or fraudulent, and which may apply to us for reasons including providing coding and billing advice to customers;

- the federal Health Insurance Portability and Accountability Act of 1996, which prohibits executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters and which also imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information;

- 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 other healthcare professionals and healthcare organizations 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 payer, including commercial insurers, state transparency laws 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.

If our operations are found to be in violation of any of the laws described above or any governmental regulations that apply to us, we will be subject to penalties, including civil and criminal penalties, damages, fines and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could adversely affect our ability to operate our business and our financial results.

In connection with the commercial launch of EXONDYS 51, we have initiated our compliance program and are in the process of assembling an experienced compliance team that will continue to develop 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.

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

The U.S. government and individual states are aggressively pursuing healthcare reform, as evidenced by the passing of the Patient Protection and Affordable Care Act, as modified by the Health Care and Education Reconciliation Act of 2010. These healthcare reform laws contain several cost containment measures that could adversely affect our future revenue, including, for example, increased drug rebates under Medicaid for brand name prescription drugs,

extension of Medicaid rebates to Medicaid managed care plans, and extension of so-called 340B discounted pricing on pharmaceuticals sold to certain healthcare providers. Additional provisions of the healthcare reform laws that may negatively affect our future revenue and prospects for profitability include the assessment of an annual fee based on our proportionate share of sales of brand name prescription drugs to certain government programs, including Medicare and Medicaid, as well as mandatory discounts on pharmaceuticals sold to certain Medicare Part D beneficiaries. 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.

In addition to government efforts in the U.S., foreign jurisdictions as well as private health insurers and managed care plans are likely to continue challenging manufacturers' ability to obtain reimbursement, as well as the level of reimbursement, for pharmaceuticals and other healthcare-related products and services. These cost-control initiatives 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 financial 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.

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

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

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

We have historically relied on U.S. government contracts and awards to fund and support certain development programs, including our Ebola and Marburg programs. The July 2010 U.S. Department of Defense ("DoD") contract providing funds for our Marburg program expired in July 2014, and the Ebola portion of the contract was previously terminated by the DoD in 2012 for convenience of the DoD. We are currently involved in contract wind-down activities and may be subject to additional government audits prior to collecting final cost reimbursements and fees owed by the government. If we are not able to complete such audits or other government requirements successfully, then the government may withhold some or all of the currently outstanding amounts owed to us. We may explore and evaluate options to continue advancing the development of our Ebola and Marburg product candidates, which may or may not include funding through U.S. government programs. As a result of government budgetary cuts, appropriations and sequestration, among other reasons, the viability of the government and its agencies as a partner for further development of our Ebola and Marburg programs, or other programs, is uncertain. The options for us to further develop product candidates that were previously developed under contracts with the U.S. government with third parties may be limited or difficult in certain respects given that, after termination or expiration of a U.S. government contract, the government has broad license rights in intellectual property developed under such contract. Therefore, the U.S. government may have the right to develop all or some parts of product candidates that we have developed under a U.S. government contract after such contract has terminated or expired.

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

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

- recruit and retain employees, consultants or contractors with the required level of expertise;

- recruit and retain sufficient patients needed to conduct a clinical trial;

- enroll and retain participants, which is a function of many factors, including the size of the relevant population, the proximity of participants to clinical sites, activities of patient advocacy groups, the eligibility criteria for the trial, the existence of competing clinical trials, the availability of alternative or new treatments, side effects from the therapy, lack of efficacy, personal issues and ease of participation;

- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the contract research organizations ("CROs") involved in the clinical trial;

- negotiate contracts and other related documents with clinical trial parties and IRBs, such as informed consents, CRO agreements and site agreements, which can be subject to extensive negotiations that could cause significant delays in the clinical trial process, with terms possibly varying significantly among different trial sites and CROs and possibly subjecting the Company to various risks;

- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;

- manage or resolve unforeseen adverse side effects during a clinical trial;

- conduct the clinical trials in a cost-effective manner, including managing foreign currency risk in clinical trials conducted in foreign jurisdictions and cost increases due to unforeseen or unexpected complications such as enrollment delays, or needing to outsource certain Company functions during the clinical trial; and

- execute clinical trial designs and protocols approved by regulatory authorities without deficiencies.

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

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

We incurred an operating loss of $178.3 million for the nine months ended September 30, 2016. Our accumulated deficit was $1.1 billion as of September 30, 2016. Although we launched EXONDYS 51 in the U.S. in September 2016, we believe that it will take us some time to attain profitability and positive cash flow from operations. Substantially all of our revenue to date has been derived from research and development contracts with the DoD, the last of which expired in July 2014. We have not yet generated any revenue from product sales and have generally incurred expenses related to research and development of our technology 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:

- launch and commercialize EXONDYS 51 in the U.S.;

- establish our sales, marketing and distribution capabilities;

- continue our research, pre-clinical and clinical development of our product candidates;

- respond to and satisfy requests and requirements from regulatory authorities in connection with development and potential approval of our product candidates;

- initiate additional clinical trials for our product candidates;

- seek marketing approvals for our product candidates that successfully complete clinical trials;

- acquire or in-license other product candidates;

- maintain, expand and protect our intellectual property portfolio;

- increase manufacturing capabilities including capital expenditures related to our real estate facilities and entering into manufacturing agreements;

- hire additional clinical, quality control and scientific personnel; and

- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

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

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

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

timing relating to securing regulatory approvals and obtaining patent rights, regulatory changes, competitive and technological developments in the market, regulatory decisions, and any commercialization expenses related to any product sales, marketing, manufacturing and distribution. An unforeseen change in these factors, or others, might increase our need for additional capital.

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

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

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

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

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

- requiring the Company to maintain pledged cash in favor of MidCap Financial equal to but not less than the lesser of the outstanding term loans or $15.0 million;

- limiting our flexibility in planning for, or reacting to, changes in our business and our industry;

- placing us at a competitive disadvantage compared to our competitors who have less debt or competitors with comparable debt at more favorable interest rates;

- limiting our ability to borrow additional amounts for working capital, capital expenditures, research and development efforts, acquisitions, debt service requirements, execution of our business strategy and other purposes; and

- resulting in an acceleration of the maturity of such term loans upon the occurrence of a material adverse change or another default under the credit and security agreement.

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

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

Our consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the U.S. The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. Such estimates and judgments include those related to revenue recognition, accrued expenses and assumptions in the valuation of stock-based compensation. We base our estimates on historical experience, facts and circumstances known to us and on various other assumptions that we believe to be reasonable under the circumstances. We cannot provide assurances, however, that our estimates, or the assumptions underlying them, will not change over time or otherwise prove inaccurate. If this is the case, we may

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

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

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

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

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

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

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

We anticipate continued growth in our business operations due, in part, to the commercialization of EXONDYS 51. This future growth could create a strain on our organizational, administrative and operational infrastructure. Our ability to manage our growth properly and maintain compliance with all applicable rules and regulations will require us to continue to improve our operational, legal, financial and management controls, as well as our reporting systems and procedures. We may not be able to build the management and human resources and infrastructure necessary to

support the growth of our business. The time and resources required to implement systems and infrastructure that may be needed to support our growth is uncertain, and failure to complete implementation in a timely and efficient manner could adversely affect our operations.

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

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

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

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

We may not be able to obtain and maintain patent protection for our product or product candidates necessary to prevent competitors from commercializing competing product candidates. Our patent rights might be challenged, invalidated, circumvented or otherwise not provide any competitive advantage, and we might not be successful in challenging the patent rights of our competitors through litigation or administrative proceedings. For example, in July 2014, the Patent Trial and Appeal Board (the "PTAB") of the United States Patent and Trademark Office ("USPTO") declared patent interferences between certain patents held by Sarepta (under license from the University of Western Australia, "UWA") and patent applications held by BioMarin (under license from Academisch Ziekenhuis Leiden, "AZL") related to exon 51 and exon 53 skipping therapies designed to treat DMD. In particular, the PTAB declared Interference No. 106,008, which identifies Sarepta's/UWA's U.S. Patent Nos. 7,807,816 and 7,960,541, both covering EXONDYS 51, as interfering with BioMarin's/AZL's U.S. Application No. 13/550,210. The PTAB also declared Interference No. 106,007, which identifies Sarepta's/UWA's U.S. Patent No. 8,455,636, covering SRP-4053, as interfering with BioMarin's/AZL's U.S. Application No. 11/233,495. In September 2014, the PTAB declared a third patent interference relating to certain methods concerning the exon 51 skipping therapies that are the subject of Interference No. 106,008. In particular, the PTAB declared Interference No. 106,013, which identifies Sarepta's/UWA's U.S. Patent No. 8,486,907, which covers certain methods of using EXONDYS 51, as interfering with BioMarin's/AZL's U.S. Application No. 14/198,992. In addition, in a September 2014 Order in Interference No. 106,007, the PTAB authorized us to file a motion with the PTAB, which we filed in November 2014, requesting the declaration of a fourth interference relating to certain methods concerning the exon 53 skipping therapies that are the subject of Interference No. 106,007, including SRP-4053, and between Sarepta's/UWA's U.S. Patent No. 8,455,636 and BioMarin's/AZL's U.S. Application No. 14/248,279. In Interference No. 106,013, we received notice on September 29, 2015 that the PTAB had issued a decision that resulted in a judgment against Sarepta and an order for the cancellation of Sarepta's/UWA's U.S. Patent No. 8,486,907 that covers certain methods of using EXONDYS 51 thereby leaving open the possibility of BioMarin's/AZL's competing U.S. Application No. 14/198,992 to issue and, if so, potentially provide a basis for BioMarin to allege that EXONDYS 51 infringes a patent granting from this application. We filed a Request for Rehearing that requests the PTAB to continue this interference, and the PTAB denied our Request on December 29, 2015. We appealed this decision to the U.S. Court of Appeals for the Federal Circuit on March 28, 2016, and this appeal was docketed as Case Nos. 16-1937 (lead) & 16-2016 (consolidated). In Interference No. 106,007, the PTAB entered a judgment on the motions on April 29, 2016 to end this interference between U.S. Patent No. 8,455,636 held by Sarepta (under license from UWA) and U.S. Application No. 11/233,495 held by BioMarin (under license from AZL) related to exon 53 skipping therapies, including SRP-4053, designed to treat DMD. The PTAB ordered: (i) the final refusal of all claims of BioMarin's/AZL's U.S. Application No. 11/233,495, with the exception of claim 77; and (ii) cancellation of all claims in Sarepta's/UWA's U.S. Patent No. 8,455,636, in each case based on its decision of various motions. The PTAB denied our motion filed in November 2014 requesting the declaration of a fourth interference relating to certain methods concerning the exon 53 skipping therapies that are the subject of this Interference No. 106,007, including SRP-4053, and between Sarepta's U.S. Patent No. 8,455,636 and BioMarin's U.S. Application No. 14/248,279, thereby leaving open the possibility of

BioMarin's/AZL's competing U.S. Application No. 14/248,279 to issue and, if so, potentially provide a basis for BioMarin to allege that our product candidate, SRP-4053, infringes a patent granting from this application. BioMarin appealed the decision from Interference No. 106,007 to the U.S. Court of Appeals for the Federal Circuit on June 28, 2016, and this appeal was docketed as Case No. 16-2262 and designated by the Court as a companion case to the exon 51 methods interference appeal (Case No. 16-1937). On September 20, 2016, the PTAB issued a judgment in Interference No. 106,008 against BioMarin/AZL and ordered the final result of all claims of AZL's application, U.S. Application No. 13/550,210. BioMarin/AZL may request rehearing before the PTAB and/or appeal the decision to the U.S. Court of Appeals for the Federal Circuit. We cannot make any assurances about the outcome of any rehearing decisions or appeals of any of these three interferences. Any adverse rulings on rehearing or appeal could come at any time and, if negative, could adversely affect our business and result in a decline in our stock price. If final resolution of the interference and related appeals are not in our favor, then the Sarepta/UWA patents involved in the interference, any other Sarepta/UWA patents or applications also found to be interfering, and any other Sarepta/UWA patents or applications may be invalidated or subject to invalidation, and as a result, we may not have any patent-based exclusivity available for our product or product candidates, which may have a material negative impact on our business plans. In addition, if final resolution of the interference or related appeals are not in our favor, the USPTO may issue the BioMarin/AZL patent applications resulting in the grant of one or more patents that may provide a basis for BioMarin to allege that EXONDYS 51 and/or our product candidate, SRP-4053, infringe such patents. In addition, the interference, appeals and any

subsequent litigation may require significant financial resources that we may have planned to spend on other Company objectives, resulting in delays or other negative impacts on such other objectives. In addition, BioMarin may continue to evaluate other opportunities to challenge our intellectual property rights or seek to broaden their patent positions in an attempt to cover our product candidates in the U.S. and in other jurisdictions. We are also aware of certain pending and granted claims that are held by BioMarin in Japan, Europe and certain other countries that may provide the basis for BioMarin or other parties to assert that EXONDYS 51 infringes on such claims. Because we have not yet initiated an invalidation proceeding in these countries, the outcome and timing of any such proceeding cannot be predicted or determined as of the date of this report.

As a matter of public policy, there might be significant pressure on governmental bodies to limit the scope of patent protection or impose compulsory licenses for disease treatments that prove successful. Additionally, jurisdictions other than the U.S. might have less restrictive patent laws than the U.S., giving foreign competitors the ability to exploit these laws to create, develop and market competing products. The USPTO and patent offices in other jurisdictions have often required that patent applications concerning pharmaceutical and/or biotechnology-related inventions be limited or narrowed substantially to cover only the specific innovations exemplified in the patent application, thereby limiting the scope of protection against competitive challenges. Accordingly, even if we or our licensors are able to obtain patents, the patents might be substantially narrower than anticipated.

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

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

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

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

important commercial markets.

If EXONDYS 51 or our product candidates or technologies infringe enforceable proprietary rights of others, we could incur substantial costs and may have to:

- obtain rights or licenses from others, which might not be available on commercially reasonable terms or at all;
- abandon development of an infringing product candidate;
- redesign EXONDYS 51, product candidates or processes to avoid infringement;
- pay damages; and/or
- defend litigation or administrative proceedings which might be costly whether we win or lose, and which could result in a substantial diversion of financial and management resources.

Any of these events could substantially harm our potential earnings, financial condition and operations. BioMarin has rights to patent claims that, absent a license, may preclude us from commercializing EXONDYS 51 in several jurisdictions. BioMarin has

rights to European Patent No. EP 1619249, for example. We opposed this patent in the Opposition Division of the European Patent Office ("EPO"), and the Opposition Division maintained certain claims of this patent relating to the treatment of DMD by skipping dystrophin exons 51 and 46, which may provide a basis to maintain that commercialization of EXONDYS 51 in a European country where BioMarin has a patent corresponding to EP 1619249 would infringe on such patent. Both we and BioMarin have appealed the Opposition Division decision, submitted briefs in support of our respective positions and have also submitted responses to each other's briefs. BioMarin filed arguments with the EPO in response to Sarepta's previously filed briefs. The Opposition Division decision, if maintained at the appeals level, could have a substantial negative effect on our business and leaves open the possibility that BioMarin or other parties that have rights to such patent could assert that EXONDYS 51 infringes on such patent in a relevant European country. The timing and outcome of the appeal cannot be predicted or determined as of the date of this report. If as part of any appeal before the EPO we are unsuccessful in invalidating BioMarin's claims that were maintained by the Opposition Division or if claims previously invalidated by the Opposition Division are restored on appeal, our ability to commercialize both EXONDYS 51 and our therapeutic candidates could be materially impaired. Moreover, our ability to commercialize EXONDYS 51 in a European country where BioMarin has a patent related to EP 1619249 while the appeal process remains ongoing before the EPO Board of Appeals could be materially impaired. In addition, we are aware of various divisional applications relating to EP 1619249 that are being pursued by BioMarin, which are pending and in some cases are granted. Any of these granted patents can also materially impair our ability to commercialize EXONDYS 51 or our therapeutic candidates, such as SRP-4045 and SRP-4053.

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

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

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

The biotechnology and pharmaceutical industries are highly competitive and subject to significant and rapid technological change. We are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development in areas related to antisense technology and other RNA technologies, or that are developing alternative approaches to or therapeutics for the disease indications on which we are focused. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with EXONDYS 51 or our product candidates. For example, we believe that companies including Alnylam Pharmaceuticals, Inc., Ionis Pharmaceuticals, Inc. (formerly Isis Pharmaceuticals, Inc.), Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Wave Life Sciences ("Wave"), and Nippon Shinyaku Co. Ltd. share a focus on RNA-targeted drug discovery and development. Competitors with respect to EXONDYS 51 include BioMarin (which acquired Prosensa), Nippon Shinyaku, Daiichi Sankyo, Wave and Shire plc; and other companies such as PTC Therapeutics and Summit plc have also been working on DMD programs. Additionally, several

companies have entered into collaborations or other agreements for the development of product candidates, including mRNA, gene (CRIPSR and AAV, among others) and small molecule therapies that are potential competitors for therapies being developed in the muscular dystrophy, neuromuscular and rare disease space, including, but not limited to, Pfizer, Inc., Bristol-Myers Squibb, Biogen Idec, Inc., Ionis Pharmaceuticals, Inc., Alexion Pharmaceuticals, Inc., Sanofi, Eli Lilly, Alnylam, Moderna Therapeutics, Inc., Summit plc, Akashi, Catabasis, and Oxford University. Although BioMarin received a complete response letter for Kyndrisa™ (drisapersen) for the treatment of DMD in patients with mutations that are amenable to exon 51 skipping on January 14, 2016, BioMarin continues to be a competitor for us on the development of DMD exon-skipping product candidates.

On May 31, 2016, BioMarin announced the withdrawal of its market Authorization Application for Kyndrisa™ (drisapersen) in Europe and its intent to discontinue clinical and regulatory development of Kyndrisa™ and three other follow-on products, BMN 044, BMN 045 and BMN 053. If BioMarin or any of our competitors are successful in obtaining regulatory approval for any of their product candidates, it may limit our ability to gain or keep market share in the DMD space or other diseases targeted by our exon-skipping platform and product candidate pipeline.

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

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

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

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

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

Our research and development activities involve the use of hazardous materials, including organic and inorganic solvents and reagents. Accordingly, we are subject to federal, state and local laws and regulations governing the use, storage, handling, manufacturing, exposure to and disposal of these hazardous materials. In addition, we are subject to

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

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

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

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

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

Risks Related to Our Common Stock

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

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

- the commercial performance of EXONDYS 51 in the U.S.;
- the timing of our submissions to regulatory authorities and regulatory decisions and developments;
- positive or negative clinical trial results or regulatory interpretations of data collected in clinical trials conducted by us, our strategic partners, our competitors or other companies with investigational drugs targeting the same, similar or related diseases to those targeted by us;
- delays in beginning and completing pre-clinical and clinical studies for potential product candidates;
- delays in entering or failing to enter into strategic relationships with respect to development and/or commercialization of EXONDYS 51 or our product candidates or entry into strategic relationships on terms that are not deemed to be favorable to our Company;
- technological innovations, product development or additional commercial product introductions by ourselves or competitors;
- changes in applicable government regulations or regulatory requirements in the approval process;

- developments concerning proprietary rights, including patents and patent litigation matters, such as developments in the interferences declared by the USPTO, including in the near term any outcomes of ongoing interference proceedings and over the longer term the outcomes from any related appeals;

- public concern relating to the commercial value, efficacy or safety of any of our products;

- our ability to obtain funds, through the issuance of equity or equity linked securities or incurrence of debt, or other corporate transactions;

- comments by securities analysts;

- developments in litigation such as the stockholder lawsuits against us;

- changes in senior management; or

- general market conditions in our industry or in the economy as a whole.

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

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

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

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

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

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

Our quarterly operating results have fluctuated in the past, and we believe they will continue to do so in the future. Our operating results may fluctuate due to the variable nature of our revenue and research and development expenses. Likewise, our research and development expenses may experience fluctuations as a result of the timing and magnitude of expenditures incurred in support of our proprietary drug development programs. In one or more future periods, our results of operations may fall below the expectations of securities analysts and investors. In that event, the market price of our common stock could decline.

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

As of September 30, 2016, there were approximately 54.4 million shares of common stock outstanding and outstanding awards to purchase 6.3 million shares of common stock under various incentive stock plans. Additionally, as of September 30, 2016, there were approximately 2.8 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan, approximately 0.3 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan and approximately 1.1 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan. We may issue additional common stock and warrants from time to time to finance our operations. We may also issue additional shares to fund potential acquisitions or in connection with additional stock options or other equity awards granted to our employees, officers, directors and consultants under our Amended and Restated 2011 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. The issuance of additional shares of common stock or warrants to purchase common stock and the perception that such issuances may occur or exercise of outstanding warrants or options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

None.

Item 6. Exhibits.

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

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: November 7, 2016 By: /s/ EDWARD KAYE, MD
Edward Kaye, MD
President, Chief Executive Officer and Chief Medical Officer

(Principal Executive Officer)

Date: November 7, 2016 By: /s/ SANDESH MAHATME
Sandesh Mahatme
Senior Vice President, Chief Financial Officer

(Principal Financial and Accounting Officer)

EXHIBIT INDEX

Exhibit		Incorporated by Reference to Filings Indicated				
			File		Filing	Provided
Number	Exhibit Description	Form	No.	Exhibit	Date	Herewith
4.1	Sarepta Therapeutics, Inc. Amended and Restated 2011 Equity Incentive Plan	8-K	001-14895	10.1	07/01/2016	
4.2	Sarepta Therapeutics, Inc. Amended and Restated 2013 Employee Stock Purchase Plan	8-K	001-14895	10.2	07/01/2016	
10.1	Executive Employment Agreement dated September 20, 2016 by and between Sarepta Therapeutics, Inc. and Edward M. Kaye, M.D.					X
10.2	License and Collaboration Agreement by and between Summit (Oxford) Ltd. and Sarepta Therapeutics, Inc. dated October 3, 2016.					X
31.1	Certification of the Company's Interim Chief Executive Officer, Edward Kaye, MD, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company's Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1*	Certification of the Company's Interim Chief Executive Officer, Edward Kaye, MD, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2*	Certification of the Company's Senior Vice President, Chief Financial Officer, Sandesh Mahatme, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	XBRL Instance Document.					X
101.SCH	XBRL Taxonomy Extension Schema Document.					X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.					X

101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.	X

*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.