Sarepta Therapeutics, Inc.
Form 10-Q
November 01, 2017

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, 2017

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
Emerging growth company		

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

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

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

Common Stock with $0.0001 par value	64,632,001
(Class)	(Outstanding as of October 26, 2017)

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, 2017	As of December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$617,630	$122,420
Short-term investments	—	195,425
Accounts receivable	24,751	5,228
Inventory	64,693	12,813
Restricted investment	—	10,695
Asset held for sale	1,501	—
Other current assets	27,033	26,895
Total current assets	735,608	373,476
Restricted cash and investments	784	784
Property and equipment, net of accumulated depreciation of $34,677 and $30,346 as of September 30, 2017 and December 31, 2016, respectively	38,872	37,801
Intangible assets, net of accumulated amortization of $3,762 and $3,134 as of September 30, 2017 and December 31, 2016, respectively	14,029	8,076
Other non-current assets	10,988	3,967
Total assets	$800,281	$424,104
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$5,317	$29,690
Accrued expenses	55,752	31,016
Current portion of long-term debt	4,732	10,108
Deferred revenue	3,303	3,303
Other current liabilities	1,366	1,305
Total current liabilities	70,470	75,422
Long-term debt	26,550	6,042

Deferred rent and other	6,105	5,949
Total liabilities	103,125	87,413
Commitments and contingencies (Note 18)		
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; 64,567,418 and 54,759,234 issued and outstanding at September 30, 2017 and December 31, 2016, respectively	6	5
Additional paid-in capital	1,890,172	1,503,126
Accumulated other comprehensive loss	(12)	(120)
Accumulated deficit	(1,193,010)	(1,166,320)
Total stockholders' equity	697,156	336,691
Total liabilities and stockholders' equity	$800,281	$424,104

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		For the Nine Months Ended	
	September 30,		September 30,	
	2017	2016	2017	2016
Revenues:				
Product, net	$ 45,954	$ —	$ 97,307	$ —
Total revenues	45,954	—	97,307	—
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	3,078	—	3,807	—
Research and development	34,239	34,349	122,266	117,523
Selling, general and administrative	28,176	22,184	90,461	60,812
Settlement and license charges	25,588	—	28,427	—
Amortization of in-licensed rights	780	—	837	—
Total cost and expenses	91,861	56,533	245,798	178,335
Operating loss	(45,907)	(56,533)	(148,491)	(178,335)
Other income (loss):				
Gain from sale of Priority Review Voucher	—	—	125,000	—
Interest income (expense) and other, net	184	(209)	703	(478)
Total other income (loss)	184	(209)	125,703	(478)
Loss before income tax expense	(45,723)	(56,742)	(22,788)	(178,813)
Income tax expense	2,011	—	3,902	—
Net loss	(47,734)	(56,742)	(26,690)	(178,813)
Other comprehensive income (loss):				
Unrealized gain (loss) on cash equivalents and short-term investments	26	(1)	108	111
Total other comprehensive income (loss)	26	(1)	108	111
Comprehensive loss	$ (47,708)	$ (56,743)	$ (26,582)	$ (178,702)
Net loss per share - basic and diluted	$ (0.78)	$ (1.18)	$ (0.47)	$ (3.83)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	61,528	48,254	57,166	46,709

See accompanying notes to unaudited condensed consolidated financial statements.

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SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(unaudited, in thousands)

	For the Nine Months Ended September 30,	
	2017	2016
Cash flows from operating activities:		
Net loss	$ (26,690)	$ (178,813)
Adjustments to reconcile net loss to cash flows from operating activities:		
Gain from sale of Priority Review Voucher	(125,000)	—
Depreciation and amortization	5,968	3,947
(Accretion of discount) amortization of premium on available-for-sale securities and non-cash interest	(144)	473
Loss on disposal of assets	792	45
Stock-based compensation	23,099	23,093
Non-cash restructuring expenses	—	504
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable	(19,523)	(9)
Net increase in inventory	(51,880)	(2,921)
Net increase in other assets	(7,319)	(8,203)
Net decrease in accounts payable, accrued expenses, deferred revenue and other liabilities	(241)	(2,703)
Net cash used in operating activities	(200,938)	(164,587)
Cash flows from investing activities:		
Purchase of property and equipment	(8,101)	(2,427)
Purchase of intangible assets	(8,591)	(1,093)
Purchase of available-for-sale securities	(100,348)	—
Proceeds from sale of Priority Review Voucher	125,000	—
Maturity of restricted investment	10,695	—
Maturity and sale of available-for-sale securities	296,225	112,101
Net cash provided by investing activities	314,880	108,581
Cash flows from financing activities:		
Proceeds from July 2017 Term Loan (defined in Note 12), net of cash debt issuance costs	29,620	—
Proceeds from revolving line of credit	24,000	—
Payments on June 2015 Term Loan (defined in Note 12) and mortgage loans	(15,081)	(5,076)
Payments on revolving line of credit	(23,008)	—
Proceeds from sales of common stock, net of offering costs	353,959	364,951
Proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program	11,779	10,967

Net cash provided by financing activities	381,269	370,842
Increase in cash and cash equivalents	495,211	314,836
Cash, cash equivalents and restricted cash:		
Beginning of period	122,556	80,440
End of period	617,767	395,276
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 924	$ 1,199
Supplemental schedule of non-cash investing activities and financing activities:		
Shares withheld for taxes	$ 1,791	$ 1,955
Intangible assets included in accrued expenses	$ 258	$ 1,230
Reclassification of software licenses	$ 204	$ —
Property and equipment reclassified to asset held for sale	$ 1,529	$ —
Accrual for debt issuance costs related to the term loans	$ 600	$ 400
Accrual for offering costs related to equity offerings	$ 25	$ 222
Property and equipment included in accrued expenses	$ 385	$ —

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

1. BUSINESS

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

In November 2016, the Company submitted a marketing authorization application ("MAA") for eteplirsen to the European Medicine Agency ("EMA") and the application was validated in December 2016. The Company continues to work with the EMA during their review process and anticipate they will complete their review and make a final decision on the approvability of the Company's MAA for eteplirsen in the first half of 2018.

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

As of September 30, 2017, the Company had approximately $618.4 million of cash, cash equivalents and investments, consisting of $617.6 million of cash and cash equivalents and $0.8 million of restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments as of the date of the issuance of this report is sufficient to fund its current operational plan for at least the next twelve months, though it may pursue additional cash resources through public or private financings, seek additional government funding and establish collaborations with or license its technology to other companies.

2. SIGNIFICANT ACCOUNTING POLICIES AND RECENT ACCOUNTING PRONOUNCEMENTS

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States ("U.S. GAAP"), reflect the accounts of

Sarepta Therapeutics, Inc. and its wholly-owned subsidiaries. All intercompany transactions between and among its consolidated subsidiaries have been eliminated. Management has determined that the Company operates in one segment: discovering, developing, manufacturing and delivering therapies to patients for the treatment of rare neuromuscular diseases.

Estimates and Uncertainties

The preparation of the unaudited condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, equity, revenue, expenses and the disclosure of contingent assets and liabilities. Actual results could differ from those estimates. Significant items subject to such estimates and assumptions include revenue recognition, inventory, valuation of stock-based awards, research and development expenses and income tax.

Concentration of Credit Risk

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

As of September 30, 2017, the majority of the Company’s accounts receivable have arisen from product sales in the U.S. and all customers have standard payment terms which generally require payment within 30 to 60 days. Three individual customers accounted for 50%, 32% and 18% of net U.S. product revenues and 66%, 21% and 13% of accounts receivable from product sales,

respectively. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profile. As of September 30, 2017, the Company believes that such customers are of high credit quality.

As of September 30, 2017, the Company's money market funds, commercial paper and government and governmental agency bonds were concentrated at two financial institutions, which potentially exposes the Company to credit risks. However, the Company does not believe that there is significant risk of non-performance by the financial institutions.

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, 2016.

In November 2016, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") No. 2016-18, "Statement of Cash Flows: Restricted Cash". The amendments in this update requires amounts generally described as restricted cash and restricted cash equivalents be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. ASU No. 2016-18 will be effective for fiscal years beginning after December 15, 2017, with early adoption permitted. The Company elected to early adopt this guidance as of January 1, 2017. This guidance was applied using a retrospective transition method for each period and, accordingly, the Company included approximately $0.1 million of restricted cash in cash and cash equivalents as of the beginning and ending periods in the accompanying unaudited condensed consolidated statements of cash flows.

During the second quarter of 2017, the Company granted its new CEO 3,300,000 options with service and market conditions. A market condition relates to the achievement of a specified price of the Company's common stock, a specified amount of intrinsic value indexed to the Company's common stock or a specified price of the Company's common stock in terms of other similar equity shares. The grant date fair value for the options with service and market conditions is determined by a lattice model with Monte Carlo simulations and, with consideration given to estimated forfeitures, is recognized as stock-based compensation expense on a straight-line basis over the service period.

There have not been any other material changes to the Company's accounting policies as of September 30, 2017.

Recent Accounting Pronouncements

In May 2017, the FASB issued ASU No. 2017-09, "Compensation – Stock Compensation (Topic 718): Scope of Modification Accounting". The amendments in this update provide guidance about which changes to the terms or conditions of a stock-based payment award requires an entity to apply modification accounting in Topic 718. ASU No. 2017-09 will be effective for fiscal years beginning after December 15, 2017, with early adoption permitted. The Company elected to early adopt this guidance as of June 30, 2017 and determined that the adoption of this guidance does not have any impact on its consolidated financial statements.

In August 2016, the FASB issued ASU No. 2016-15, "Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments". The amendments in this update clarify how certain cash receipts and cash payments are presented and classified in the statement of cash flows. ASU No. 2016-15 will be effective for fiscal years beginning after December 15, 2017, with early adoption permitted. As of September 30, 2017, 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 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. In transition, lessees and lessors are required to recognize and measure leases at the beginning of the earliest period presented using a modified retrospective approach. ASU No. 2016-02 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The adoption of this standard is expected to have an impact on the amount of the Company’s assets and liabilities. As of September 30, 2017, 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 May 2014, the FASB issued ASU No. 2014-09, “Revenue from Contracts with Customers (Topic 606)”. This ASU supersedes the revenue recognition requirements in Accounting Standards Codification Topic 605, “Revenue Recognition”. Under the new guidance, a company is required to recognize revenue when it transfers goods or renders services to customers at an amount that it expects to be entitled to in exchange for these goods or services. The new standard allows for either a full retrospective with or without practical expedients or a retrospective with a cumulative catch upon adoption transition method. This guidance was originally intended to be effective for the fiscal years beginning after December 15, 2016, with early adoption not permitted. In August 2015, the

FASB issued ASU No. 2015-14, “Deferral of the Effective Date”, which states that the mandatory effective date of this new revenue standard will be delayed by one year, with early adoption only permitted in fiscal year 2017. During the second quarter of 2016, the FASB issued three amendments to the new revenue standard to address some application questions: ASU No. 2016-10, “Identifying Performance Obligations and Licensing”, ASU No. 2016-11, “Rescission of SEC Guidance Because of Accounting Standards Updates 2014-09”, and ASU No. 2016-12, “Narrow-Scope Improvements and Practical Expedients”. In December 2016, the FASB issued ASU No. 2016-20, “Technical Corrections and Improvements to Topic 606, Revenue from Contracts with Customers”, which amends certain narrow aspects of the guidance issued in ASU 2014-09 including guidance related to the disclosure of remaining performance obligations and prior-period performance obligations, as well as other amendments to the guidance on loan guarantee fees, contract costs, refund liabilities, advertising costs and the clarification of certain examples. These three amendments will be effective upon adoption of Topic 606. The Company is currently reviewing the new standards as compared to its current accounting policies with respect to its product revenues and a review of its customer contracts is also in process. During the last quarter of 2017, the Company plans to finalize its review of product revenues in the U.S. as well as revenue streams from its MAPs to determine the impact that this standard may have on its results of operations, financial position and disclosures. As of September 30, 2017, the Company has determined that it will utilize the full retrospective adoption method but has not finalized the effect that the adoption of this guidance will have on its consolidated financial statements.

Reclassification

The Company has revised the presentation as well as the captions of certain accrued expenses in Note 11, Accrued Expenses to the unaudited condensed consolidated financial statements to conform to the current period presentation. “Product revenue related reserves” of $0.3 million as of December 31, 2016 has been reclassified from “Other” of $3.6 million and presented separately in the accrued expenses table. The reclassification had no impact on total current liabilities or total liabilities.

Subsequent Events

The Company evaluated subsequent events from September 30, 2017 through the date of issuance of this report and concluded that no subsequent events have occurred that would require recognition or disclosure in the unaudited condensed consolidated financial statements.

3. LITIGATION SETTLEMENT AND LICENSE AGREEMENTS

In July 2017, the Company and the University of Western Australia (“UWA”) entered into a settlement agreement with BioMarin Leiden Holding BV, its subsidiaries BioMarin Nederlands BV and BioMarin Technologies BV (collectively, “BioMarin”). On the same day, the Company entered into a license agreement with BioMarin and Academisch Ziekenhuis Leiden (“AZL”) (collectively with the Company, UWA and BioMarin, the “Settlement Parties”). Under these agreements, BioMarin agreed to provide the Company with an exclusive license to certain intellectual property with an option to convert the exclusive license into a co-exclusive license and the Settlement Parties agreed to stop most existing efforts to continue with ongoing litigation and opposition and other administrative proceedings concerning BioMarin’s intellectual property. Under terms of the agreements, the Company agreed to make total up-front payments of $35.0 million upon execution of the agreements, consisting of $20.0 million under the settlement agreement and $15.0 million under the license agreement. Additionally, the Company may be liable for up to approximately $65.0 million in regulatory and sales milestones for eteplirsen as well as exon 45 and exon 53 skipping product candidates. BioMarin will also be eligible to receive royalty payments, ranging from 4% - 8%, for exon 51 skipping products, exon 45 skipping products and exon 53 skipping products. The royalty terms under the license

agreement will expire in December 2023 in the U.S. and September 2024 in the EU.

In July 2017, the Company made the cash payment of $35.0 million to BioMarin. Accordingly, as of September 30, 2017, the Company has recorded an intangible asset in the U.S. of $6.6 million on its unaudited condensed consolidated balance sheet. For the three and nine months ended September 30, 2017, the Company recorded $25.6 million and $28.4 million settlement and license charges, respectively, in its unaudited condensed consolidated statements of operations and comprehensive loss.

The intangible asset represents the fair value of the U.S. license to BioMarin's intellectual property related to EXONDYS 51, which was determined by an income-based approach, and will be amortized on a straight-line basis over the remaining life of the patent. For both the three and nine months ended September 30, 2017, the Company recognized intangible asset amortization expense and royalties of approximately $0.8 million and $2.3 million, respectively. The royalties are included in cost of sales in the Company's unaudited condensed consolidated statements of operations and comprehensive loss.

4. GAIN FROM SALE OF PRIORITY REVIEW VOUCHER

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

5. 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, 2017			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$360,612	$360,612	$—	$ —
Commercial paper	83,506	—	83,506	—
Government and government agency bonds	113,934	—	113,934	—
Certificates of deposit	648	648	—	—
Total assets	$558,700	$361,260	$197,440	$ —

	Fair Value Measurement as of December 31, 2016			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$ 1,147	$ 1,147	$ —	$ —
Commercial paper	69,304	—	69,304	—
Government and government agency bonds	105,287	—	105,287	—
Corporate bonds	20,834	—	20,834	—
Certificates of deposit	11,343	11,343	—	—
Total assets	$ 207,915	$ 12,490	$ 195,425	$ —

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, 2017.

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.

6. 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. There were no available-for-sale securities as of September 30, 2017. The weighted average maturity of the Company's available-for-sale securities as of December 31, 2016 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, 2017			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$420,190	$ —	$ —	$420,190
Commercial paper	83,516	—	(10)	83,506
Government and government agency bonds	113,936	1	(3)	113,934
Total assets	$617,642	$ 1	$ (13)	$617,630
As reported:				
Cash and cash equivalents	$617,642	$ 1	$ (13)	$617,630
Total assets	$617,642	$ 1	$ (13)	$617,630

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

7. ACCOUNTS RECEIVABLE AND RESERVES FOR PRODUCT SALES

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

The accounts receivable from product sales represents receivables due from the Company's specialty distributor and specialty pharmacies. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profiles. The Company provides reserves against trade receivables for estimated losses that may result from a customer's inability to pay. Amounts determined to be uncollectible are written-off against the established reserve. As of September 30, 2017, the credit profiles for the Company's customers are deemed to be in good standing and write-offs of accounts receivable are not considered necessary. Historically, no accounts receivable amounts related to government research contracts and other grants have been written off and, thus, an allowance for doubtful accounts receivable related to government research contracts and other grants is not considered necessary.

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

	As of September 30, 2017	As of December 31, 2016
	(in thousands)	
Product sales, net of reserves	$23,822	$ 4,002
Government contract receivables	929	1,226
Total accounts receivable	$24,751	$ 5,228

The balance for government contract receivables for both periods presented is subject to government audit and will not be collected until the completion of the audit. The decrease in government contract receivables is related to contract finalization and subsequent collection of the European Union SKIP-NMD Agreement related to the Company's exon 53 product candidate.

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

	Chargebacks	Rebates	Prompt Pay	Other Accruals	Total
	(in thousands)				
Balance, as of December 31, 2016	$1	$238	$ —	$ 67	$306
Provision	3,760	4,270	78	772	8,880
Payments/credits	(3,330)	(660)	(52)	(592)	(4,634)
Balance, as of September 30, 2017	$431	$3,848	$ 26	$ 247	$4,552

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

	As of September 30, 2017	As of December 31, 2016
	(in thousands)	
Reduction to accounts receivable	$457	$ 1
Component of accrued expenses	4,095	305
Total reserves	$4,552	$ 306

8. INVENTORY

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

	As of September 30, 2017	As of December 31, 2016
	(in thousands)	
Raw materials	$44,257	$ 9,531
Work in progress	20,144	3,175
Finished goods	292	107
Total inventory	$64,693	$ 12,813

9. ASSET HELD FOR SALE

The Company owns a facility located at 1749 SW Airport Avenue, Corvallis, OR ("Airport Facility"). The Airport Facility was previously leased to an unrelated third party. In July 2016, the third party lessee terminated the lease and vacated the facility. It has been unoccupied since then. The Company set up a program and was actively marketing the Airport Facility. The Airport Facility with net book value of approximately $1.5 million was reclassified as an asset held for sale which is presented as a component of

current assets as of March 31, 2017. In August 2017, the Company entered into a purchase and sale agreement with an unrelated third-party buyer. The sale price of as well as fees related to the Airport Facility are approximately $1.5 million and $0.2 million, respectively. The transaction is scheduled to close by the end of 2017. For both the three and nine months ended September 30, 2017, the Company recognized an approximate loss of $0.2 million from the anticipated sale of the asset.

10. 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, 2017	As of December 31, 2016
	(in thousands)	
Manufacturing-related deposits and prepaids	$18,399	$23,604
Prepaid clinical and preclinical expenses	3,612	1,225
Other prepaids	4,000	1,152
Other	1,022	914
Total other current assets	$27,033	$26,895

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

	As of September 30, 2017	As of December 31, 2016
	(in thousands)	
Prepaid clinical expenses	$7,056	$3,725
Manufacturing-related deposits	3,570	—
Other	362	242
Total other non-current assets	$10,988	$3,967

11. ACCRUED EXPENSES

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

	As of September 30, 2017	As of December 31, 2016
	(in thousands)	
Accrued contract manufacturing costs	$13,438	$4,673
Accrued clinical and preclinical costs	13,123	10,033
Accrued employee compensation costs	10,560	8,748
Accrued professional fees	5,520	2,799
Product revenue related reserves	4,095	305
Accrued income taxes	3,493	—
Accrued BioMarin royalties	2,289	—
Accrued research costs	317	1,186
Other	2,917	3,272
Total accrued expenses	$55,752	$31,016

12. INDEBTEDNESS

Term Loan

In July 2017, the Company entered into an amended and restated credit agreement (the "Amended and Restated Credit and Security Agreement") which provides a term loan ("July 2017 Term Loan") of $60.0 million with MidCap Financial Trust ("MidCap"). Borrowings under the Amended and Restated Credit and Security Agreement bear interest at a rate per annum equal to 6.25%, plus the one-month London Interbank Offered Rate ("LIBOR"). In addition to paying interest on the outstanding principal under the Amended and Restated Credit and Security Agreement, the Company paid an origination fee equal to 0.50% of the amount of the term loan when advanced under the Amended and Restated Credit and Security Agreement and will be liable for a final payment fee equal to 2.00% of the amount borrowed under the Amended and Restated Credit and Security Agreement when the July

2017 Term Loan is fully repaid. Commencing on July 1, 2018, and continuing for the remaining thirty six months of the facility, the Company will be required to make monthly principal payments of approximately $0.8 million, set forth in the Amended and Restated Credit and Security Agreement, subject to certain adjustments as described therein. The facility matures in July 2021.

The Company may voluntarily prepay outstanding loans under the Amended and Restated Credit and Security Agreement at any time, provided that the Company may not prepay an amount that is less than the total of all of the credit extensions and other related obligations under the Amended and Restated Credit and Security Agreement then outstanding. In the event of a permitted prepayment, the Company is obligated to pay a prepayment fee equal to the following:

- 3.00% of the outstanding principal of such advance, if the prepayment is made within twelve months of the closing date;
- 2.00% of the outstanding principal of such advance, if the prepayment is made on or after the date which is twelve months after the closing date of such advance through the date which is twenty-four months after the closing date of such advance; and
- 1.00% of the outstanding principal of such advance, if the prepayment is made on or after the date which is twenty-four months after the closing date of such advance through the date immediately preceding the maturity date.

The Amended and Restated Credit and Security Agreement contains both affirmative and negative covenants. Affirmative covenants include government compliance, reporting requirements, maintaining property, making tax payments, maintaining insurance, cooperating during litigation, etc. Additionally, the Company is required to maintain an amount of cash and/or cash equivalents equal to not less than 75% of the sum of the outstanding principal amounts under both the Amended and Restated Credit and Security Agreement and the Revolving Credit Agreement (defined below). Negative covenants include restrictions on asset dispositions, mergers or acquisitions, indebtedness, liens, distributions, transactions with affiliates and other restrictions. The Amended and Restated Credit and Security Agreement includes customary events of default, including cross defaults and material adverse change. Additionally, the Company's failure to be compliant with the affirmative or negative covenants or make payments when they become due will result in an event of default.

After paying off certain debt issuance costs, the Company received net proceeds of $29.1 million related to the July 2017 Term Loan, $9.2 million of which was used to pay off the outstanding balance of the term loan that was taken out in June 2015 (“June 2015 Term Loan”). In connection with the July 2017 Term Loan, the Company recorded $30.0 million as long-term debt in the unaudited condensed consolidated balance sheet as of September 30, 2017. In addition, debt issuance costs of $1.1 million related to the July 2017 Term Loan were recorded as a direct deduction to the carrying value of the July 2017 Term Loan in the unaudited condensed consolidated balance sheet as of September 30, 2017. These costs are being amortized to interest expense using the effective interest method over the term of the loan.

Revolving Line of Credit

In July 2017, the Company entered into a revolving credit and security agreement (the “Revolving Credit Agreement”) which provides an aggregate revolving loan commitment of $40.0 million (which may be increased by an additional tranche of $20.0 million) with MidCap. Borrowings under the Revolving Credit Agreement bear interest at a rate of 3.95%, plus the one-month LIBOR. In addition to paying interest on the outstanding principal under the Revolving Credit Agreement, the Company paid $0.2 million of origination fee, which was 0.50% of the amount of the revolving loan. The Company recognized this origination fee as other asset and it is being amortized to interest expense over the term of the line-of-credit. Additionally, the Company is liable for unused line fees, minimum balance fees, collateral fees, deferred revolving loan original fees, etc. This facility matures in July 2021. The Company may voluntarily prepay the outstanding revolving loans under the Revolving Credit Agreement in whole or in part provided that the prepayment shall be in certain amounts as specified therein. As of September 30, 2017, the outstanding balance of the revolving line of credit is approximately $1.0 million.

Mortgage Loans

The Company has two loans outstanding which bear interest at 4.75%, mature in February 2027 and are collateralized by the Airport Facility in Corvallis, Oregon. At September 30, 2017, these loans had unpaid principal balances of $0.8 million and $0.5 million, for a total indebtedness of $1.3 million, and were presented as current portion of long-term debt on the unaudited condensed consolidated balance sheet.

For the three and nine months ended September 30, 2017, the Company recognized $0.8 million and $1.2 million of interest expense related to all outstanding loans, respectively. The following table summarizes the components of the long-term debt recorded for the period indicated:

	As of September 30, 2017	As of December 31, 2016
	(in Thousand)	
Principal amount of the 2017 Term Loan	$30,000	$ —
Principal amount of the 2015 Term Loan	—	15,000
Unamortized debt issuance expense	(1,037)	(223)
Net carrying value of term loan	28,963	14,777
Other loans	2,319	1,373
Total long-term debt	$31,282	$ 16,150

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

	Term Loan (1)	Mortgage Loans (1)	Revolver (1)	Total
	(in thousands)			
2017	$573	$ 1,307	$ 1,026	$2,906
2018	6,389	—	—	6,389
2019	11,611	—	—	11,611
2020	10,855	—	—	10,855
2021	5,980	—	—	5,980
Total Payments	$35,408	$ 1,307	$ 1,026	$37,741

(1) Includes interest

13. EQUITY OFFERINGS

In July 2017, the Company sold approximately 8.8 million shares of common stock through an underwritten public offering, including 1.2 million shares sold to the underwriters. The offering price was $42.50 per share. The Company received net proceeds of approximately $354.0 million from the offering, net of commission and offering expenses of approximately $20.0 million.

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

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

14. RESTRUCTURING

In March 2016, the Company announced a long-term plan ("Corvallis plan") to consolidate all of the Company's operations to Massachusetts as part of a strategic plan to increase operational efficiency. As part of the consolidation, research activities and some employees transitioned to the Company's facilities in Andover and Cambridge, Massachusetts. As of September 30, 2017, the relocations and terminations were completed.

The second floor and the first floor of the Corvallis facility were vacated and closed and made available for sub-leasing in December 2016 and April 2017, respectively. Using a discounted cash flow methodology and based on monthly rent payments as well as estimated sublease income, the Company recognized a total of approximately $1.5 million and $2.3 million, in restructuring expenses for the second and the first floor, respectively. As of September 30, 2017, the Company continues to be obligated to make $5.2 million of minimum lease payments and certain other contractual maintenance costs for the whole facility.

For the three months ended September 30, 2017, the restructuring expenses were de minimis. For the nine months ended September 30, 2017, the Company recorded $2.8 million of restructuring expenses, including $2.4 million related to the closure of Corvallis facility. For the three and nine months ended September 30, 2016, the Company recorded $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 tables summarize the restructuring expenses by function for the periods indicated:

	For the Three Months Ended September 30, 2017 (in thousands)			For the Three Months Ended September 30, 2016		
	Cash	Non-cash	Total	Cash	Non-cash	Total
Research and development	$10	$ —	$ 10	$628	$ 143	$771
Selling, general and administrative	3	—	3	367	126	493
Total restructuring expenses	$13	$ —	$ 13	$995	$ 269	$1,264

	For the Nine Months Ended September 30, 2017 (in thousands)			For the Nine Months Ended September 30, 2016		
	Cash	Non-cash	Total	Cash	Non-cash	Total
Research and development	$184	$ —	$184	$1,448	$ 336	$1,784
Selling, general and administrative	2,589	—	2,589	471	168	639
Total restructuring expenses	$2,773	$ —	$2,773	$1,919	$ 504	$2,423

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

	As of September 30, 2017	As of December 31, 2016
	(in thousands)	
Restructuring reserve beginning balance	$1,588	$ —
Restructuring expenses incurred during the period	2,773	3,651
Amounts paid during the period	(1,318)	(2,063)
Restructuring reserve ending balance	$3,043	$ 1,588

15. 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,			
	2017		2016		2017		2016	
	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	221,398	$ 21.07	1,050	$ 37.38	4,678,357 (1)	$ 14.49	1,214,426	$ 11.96
Restricted stock units	—	$ —	—	$ —	181,029 (3)	$ 33.03	—	$ —
Restricted stock awards	—	$ —	91,778	$ 48.94	341,500 (2)	$ 34.58	117,553	$ 41.22

(1) In June 2017, the Company granted its new CEO 3,300,000 options with service and market conditions. These options have a five-year cliff vesting schedule. The fair value of $13.48 for these options was determined by a lattice model with Monte Carlo simulations. The remaining 1,378,357 service-based options which have a weighted average grant date fair value of $16.90 have a four-year vesting schedule with 25% vesting on the first anniversary and 1/48 monthly thereafter.

(2) In June 2017, the Company granted its new CEO 335,000 restricted stock awards ("RSAs") with a fair value of $34.65. These RSAs have a four-year vesting schedule with 25% vesting on the first anniversary and 1/48 vest monthly thereafter.

(3) The Company granted executives 156,029 restricted stock units ("RSUs") with certain sales target and regulatory milestones. In June 2017, one performance condition of these RSUs was achieved. As a result, 50% of these RSUs became immediately vested and, accordingly, the Company recorded $2.5 million of stock-based compensation expenses. As of September 30, 2017, it is probable that the second performance milestone will be achieved within the required timeline. Accordingly, the Company recognized approximately $0.3 million of stock-based compensation expenses. The third performance milestone was deemed as not probable of being achieved as of September 30, 2017. If and when deemed probable that the last performance milestone may be achieved within the required time frame, the Company may recognize up to $1.1 million of stock-based compensation related to the second and third performance milestones of these grants. The remaining RSUs are service-based awards granted to the members of the board of directors.

Stock-based Compensation Expense

For the three months ended September 30, 2017 and 2016, total stock-based compensation expense was $6.9 million and $9.6 million, respectively. For both the nine months ended September 30, 2017 and 2016, total stock-based compensation expense was $23.1 million. Included in these amounts for the three and nine months ended September 30, 2017 are $(0.1) million and $2.1 million of stock-based compensation expense incurred in connection with the resignation of the Company's former CEO, respectively. The following table summarizes stock-based compensation expense by function included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2017	2016	2017	2016
	(in thousands)		(in thousands)	
Research and development	$1,812	$2,674	$5,881	$7,527
Selling, general and administrative	5,110	6,899	17,218	15,566
Total stock-based compensation expense	$6,922	$9,573	$23,099	$23,093

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,	
	2017	2016	2017	2016
	(in thousands)		(in thousands)	
Stock options	$5,420	$8,778	$17,221	$20,248
Restricted stock awards/units	954	232	4,408	689
Stock appreciation rights	—	115	—	345
Employee stock purchase plan	548	448	1,470	1,811
Total stock-based compensation expense	$6,922	$9,573	$23,099	$23,093

16. INCOME TAXES

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

For the three and nine months ended September 30, 2017, the Company recorded an income tax expense of $2.0 million and $3.9 million, respectively, representing an effective tax rate of 4.4% and 17.1%, respectively. The Company's estimated annual effective tax rate is lower than the federal statutory rate due to the jurisdictional mix of earnings and the release of valuation allowance against its federal and state tax attributes which can be used to offset current year earnings. For the three and nine months ended September 30, 2016, the Company did not record any income tax expense or benefit. The increase in the income tax expense liability as of September 30, 2017 as compared to the balance as of December 31, 2016 was due to additional state and federal income taxes payable as a result of the increase in the amount of income before income taxes. The increase in domestic income is primarily attributable to the gain on the sale of the Company's PRV to Gilead for $125.0 million in cash during the period ended March 31, 2017.

17. 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,	
	2017	2016	2017	2016
	(in thousands, except per share amounts)			
Net loss	$(47,734)	$(56,742)	$(26,690)	$(178,813)
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	61,528	48,254	57,166	46,709
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	61,528	48,254	57,166	46,709
Net loss per share - basic and diluted	$(0.78)	$(1.18)	$(0.47)	$(3.83)

* For the three and nine months ended September 30, 2017, stock options, RSAs, RSUs and stock appreciation rights (“SARs”) to purchase 9.8 million shares of the Company’s common stock were excluded from the net loss per share calculation as their effect would have been anti-dilutive. For the three and nine months ended September 30, 2016, stock options, RSAs and SARs to purchase 6.3 million shares of the Company’s common stock were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

18. COMMITMENTS AND CONTINGENCIES

Milestone Obligations

As of September 30, 2017, the Company was obligated to make up to $808.5 million of future development, up-front royalty and sales milestone payments associated with certain of its collaboration and license agreements. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory and sales milestones. As of September 30, 2017, the Company made an up-front cash payment of $35.0 million to BioMarin related to the license and settlement agreements. Accordingly, it recorded an intangible asset in the U.S. of $6.6 million on its unaudited condensed consolidated balance sheets as of September 30, 2017. For the three and nine months ended September 30, 2017, the Company recorded settlement and license charges of $25.6 million and $28.4 million, respectively, in its unaudited condensed consolidated statements of operations and comprehensive loss. Additionally, for the nine months ended September 30, 2017 and 2016, the Company recognized $22.0 million and $7.0 million milestone and up-front payments to Summit (Oxford) Ltd. and UWA, respectively, as research and development expense.

Other Funding Commitments

As of September 30, 2017, the Company has several on-going clinical studies in various clinical trial stages. Its most significant clinical trial expenditures are to contract research organizations (“CROs”). The CRO contracts are generally cancellable at its option. As of September 30, 2017, the Company has approximately $54.4 million in cancellable future commitments based on existing CRO contracts.

Litigation

In the normal course of business, the Company may from time to time be named as a party to various legal claims, actions and complaints, including matters involving securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. For example, purported class action complaints were filed against the Company and certain of its officers in the U.S. District Court for the District of Massachusetts on January 27, 2014 and January 29, 2014. The complaints were consolidated

into a single action (Corban v. Sarepta, et. al., No. 14-cv-10201) by order of the court on June 23, 2014. Plaintiffs' consolidated amended complaint, filed on July 21, 2014, asserted violations of Section 10(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act") and Securities and Exchange Commission Rule 10b-5 against the Company, and Chris Garabedian, Sandy Mahatme, and Ed Kaye ("Individual Defendants," and collectively with the Company, the "Corban Defendants"), and violations of Section 20(a) of the Exchange Act against the Individual Defendants. Plaintiffs alleged that the Corban Defendants made material misrepresentations or omissions during the putative class period of July 24, 2013 through November 12, 2013, regarding a data set for a Phase 2b study of eteplirsen and the likelihood of the FDA accepting the Company's new drug application for eteplirsen for review based on that data set. Plaintiffs sought compensatory damages and fees. On August 18, 2014, the Corban Defendants filed a motion to dismiss, which the Court granted on March 31, 2015. Plaintiffs subsequently sought leave to file a second amended complaint, which the Corban Defendants opposed. On September 2, 2015, the Court denied Plaintiffs' motion for leave to amend as futile. Plaintiffs filed a notice of appeal on September 29, 2015, seeking review of the Court's March 31, 2015 order dismissing the case and the Court's September 2, 2015 order denying leave to amend. On January 27, 2016, Plaintiffs filed in the district court a motion for relief from judgment pursuant to Federal Rule of Civil Procedure 60(b)(2), arguing that the FDA Briefing Document published on or about January 15, 2016, was material and would have changed the Court's ruling. On February 26, 2016, the First Circuit stayed the appeal pending the district court's ruling on the 60(b)(2) motion. Defendants opposed the 60(b)(2) motion, and on April 21, 2016, the Court denied Plaintiffs' motion for relief from judgment. On May 19, 2016, Plaintiffs filed a motion to alter or amend the April 21, 2016 order pursuant to Federal Rule of Civil Procedure 59(e). On May 20, 2016, the Court denied Plaintiffs' motion, and Plaintiffs filed a notice of appeal of the Court's April 21, 2016 denial of their 60(b)(2) motion and May 20, 2016 denial of their 59(e) motion. On June 13, 2016, the First Circuit granted Plaintiffs' motion to consolidate the two appeals. Oral argument took place on March 7, 2017 and the First Circuit affirmed the District Court's dismissal of this case on August 22, 2017. Plaintiffs filed a Petition for Panel Rehearing and Rehearing En Banc, which the First Circuit denied on October 11, 2017. As such, the risk of loss is not deemed probable.

Another complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 styled William Kader, Individually and on Behalf of All Others Similarly Situated v. Sarepta Therapeutics Inc., Christopher Garabedian, and Sandesh Mahatme (Kader v. Sarepta et.al 1:14-cv-14318). On March 20, 2015, Plaintiffs filed an amended complaint asserting violations of Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5 against the Company, and Chris Garabedian and Sandy Mahatme ("Individual Defendants," and collectively with the Company, the "Kader Defendants"), and violations of Section 20(a) of the Exchange Act against the Individual Defendants. Plaintiffs alleged that the Kader Defendants made material misrepresentations or omissions during the putative class period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company's data for submission of an NDA for eteplirsen and the likelihood of the FDA accepting the NDA based on that data. Plaintiffs sought compensatory damages and fees. The Kader Defendants moved to dismiss the amended complaint on May 11, 2015. On April 5, 2016, following oral argument on March 29, 2016, the Court granted Defendants' motion to dismiss. On April 8, 2016, Lead Plaintiffs filed a motion for leave to file an amended complaint, which Defendants opposed. On January 6, 2017, the Court denied Plaintiffs' motion for leave to amend and dismissed the case. Plaintiffs filed a notice of appeal on February 3, 2017. A briefing schedule was set on March 13, 2017. Appellants' brief was filed April 24, 2017. Appellee's brief was filed May 24, 2017. The Court has not yet scheduled a date for oral argument. An estimate of the possible loss or range of loss cannot be made at this time.

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

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

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

disgorgement and rescindment of allegedly excessive or unfair payments and equity grants to Mr. Garabedian and the directors, unspecified damages plus interest, a declaration that the Company's Amended and Restated 2011 Equity Plan at the 2013 annual meeting was ineffective and a revote for approved amendments, correction of misleading disclosures and plaintiff's attorney fees. The parties have agreed to a Memorandum of Understanding concerning the settlement terms and do not believe that disposition of the McDonald suit will have a material financial impact on the Company. The parties 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.

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, 2016 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:

- our continued efforts to ensure the successful commercialization of EXONDYS 51 in the U.S., expanding our global footprint, meeting or outperforming revenue projections, and maintaining our accelerated approval status, including through obtaining data from our ongoing and planned studies to determine the safety and efficacy of EXONDYS 51 and executing our plans to hire additional personnel, increase awareness on the importance of genetic testing and knowing/understanding Duchenne muscular dystrophy ("DMD") mutations, and identifying and addressing procedural barriers for patients to obtain therapy such as payor reimbursement challenges, maintaining the marketing, distribution and supply infrastructure we have built for EXONDYS 51 and our expectations regarding the timing, costs, and investments associated with these activities;
- our expectations regarding timing and the factors that will influence and our ability to obtain full approval of eteplirsen in the U.S. and in the jurisdictions we target outside of the U.S., which depends in part on data from our ongoing and planned studies demonstrating a clinical benefit and acceptable safety profile of eteplirsen, as well as our ability to (i) in the U.S., complete to the United States Food and Drug Administration's ("FDA") satisfaction of our post-marketing requirements and commitments, (ii) in the EU, successfully navigate the EU drug approval process and (iii) in jurisdictions other than the U.S. where eteplirsen could obtain regulatory approval, build the commercial, medical and other company infrastructure and product supply needed to support a successful launch;
- the potential acceptance of EXONDYS 51, and our product candidates if they receive regulatory approval, in the marketplace and the accuracy of our projections regarding the market size in each of the jurisdictions that we target;
- our ability to further secure long term supply of EXONDYS 51 and our product candidates, including our peptide-conjugated PMO ("PPMO"), to satisfy our planned commercial, managed access program ("MAP"), named-patient program and clinical needs, which could require, among other things, securing more supply of subunits, drug substance Active Pharmaceutical Ingredients ("APIs") and drug product, by negotiating and entering into additional commercial and clinical supply agreements, and further evolving or scaling up manufacturing using appropriate techniques to synthesize and purify our product candidates that meet regulatory, Company quality control and other applicable requirements;
- our expectations regarding our ability to successfully conduct or accelerate research, development, pre-clinical, clinical and post-approval trials, and our expectations regarding the timing, design and results of such trials, including the potential consistency of data produced by these trials with prior results, as well as any new data and analyses relating to the safety profile and potential clinical benefits of EXONDYS 51 and our product candidates, including SRP-4053, SRP-5051 and SRP-4045;
- our potential success in advancing the development of our follow-on exon-skipping drug candidates targeting DMD and further exploring potential funding, collaborations and other opportunities to support such development;
- the potential and advancement of our phosphorodiamidate morpholino oligomer ("PMO") chemistries, our PPMO chemistries, our other PMO-based chemistries, and our other technologies to treat DMD and other diseases and therapeutic areas that we target;
- our ability to successfully expand the global footprint of eteplirsen in jurisdictions in which we have yet to obtain or do not have any near term ability or plans to obtain a full regulatory approval, including through obtaining an approval from the European Medicines Agency ("EMA") in the EU, establishing compliant and successful MAPs, expanding our MAPs to include more countries over time, and entering into any additional distribution, service and

other contracts;

- the impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations;
- the possible impact of any competing products on the commercial success of EXONDYS 51 and our product candidates and our ability to compete against such products;

- the impact of potential difficulties in product development manufacturing for commercial or clinical supply of EXONDYS 51 or pre-clinical or clinical supply of our product candidates, including PPMO, due to potential negative factors such as failing to successfully establish and maintain the Company infrastructure necessary to support the Company's research, development and commercialization efforts;
- our expectations regarding our ability to become a leading developer and marketer of PMO-based and RNA-targeted therapeutics and commercial viability of EXONDYS 51 across various jurisdictions, as well as our product candidates, chemistries and technologies;
- our ability to enter into research, development or commercialization alliances with universities, hospitals, independent research centers, non-profit organizations, pharmaceutical and biotechnology companies and other entities for specific molecular targets or selected disease indications and our ability to selectively pursue opportunities to access certain intellectual property rights that complement our internal portfolio through license agreements or other arrangements;
- our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future;
- the extent of protection that our patents provide and our pending patent applications may provide, if patents issue from such applications, to our technologies and programs, and our ability to obtain and maintain patent protection for our technologies and programs;
- our plans and ability to file and progress to issue additional patent applications to enhance and protect our new and existing technologies and programs;
- our ability to invalidate some or all of the claims of patents issued to competitors and pending patent applications if issued to competitors, and the potential impact of those claims on the potential commercialization and continued commercialization, where authorized, of EXONDYS 51 and the potential commercialization of our product candidates, including SRP-4053, SRP-5051 and SRP-4045;
- our ability to operate our business without infringing the intellectual property rights of others;
- our estimates regarding how long our currently available cash and cash equivalents will be sufficient to finance our operations and business plans and statements about our future capital needs;
- our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;
- our ability to raise additional funds to support our business plans and strategies, including business development, and the impact of our amended and restated credit and security agreement with MidCap Financial Trust, a Delaware statutory trust, as administrative agent ("MidCap") and new revolving credit and security agreement with MidCap, on our financial condition and future operations;
- our expectations relating to potential funding from government and other sources for the development of some of our product candidates;
- the 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 expectation that Dr. Edward M. Kaye will serve us in an advisory capacity to ensure a smooth transition to our new Chief Executive Officer, Mr. Douglas S. Ingram, and expectations regarding the potential benefits the Company may inure under Mr. Ingram's leadership;
- our ability to comply with applicable environmental laws and regulations;
- the impact of the potential achievement of performance conditions and milestones relating to our stock awards; and
- our beliefs and expectations regarding milestone, royalty or other payments that could be due to third parties under existing agreements.

We undertake no obligation to update any of the forward-looking statements contained in this Quarterly Report on Form 10-Q after the date of this report, except as required by law or the rules and regulations of the U.S. Securities and Exchange Commission ("SEC"). We caution readers not to place undue reliance on forward-looking statements. Our actual results could differ materially from those discussed in this Quarterly Report on Form 10-Q. The forward-looking statements contained in this Quarterly Report on Form 10-Q, and other written and oral forward-looking statements made by us from time to time, are subject to certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements, including the risks, uncertainties and assumptions identified under the heading "Risk Factors" in this Quarterly Report on Form 10-Q.

Overview

U.S. Approval, MAA, and MAP

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

Additionally, we submitted a marketing authorization application ("MAA") for eteplirsen to the EMA in November 2016 and the application was validated in December 2016. We continue to work with the EMA during their review process, and we expect to receive a response from the EMA's Committee for Medicinal Products for Human Use on our application in the first half of 2018.

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

Our RNA-targeted Technologies

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

The original PMO structure and variations of this structure referred to herein (collectively "PMO-based") are central to our proprietary chemistry platform. Our next generation PMO-based chemistries include PPMO, PMO-X® and PMOplus®. PMO-based compounds are highly resistant to degradation by enzymes, potentially enabling robust and sustained biological activity. In contrast to other RNA-targeted therapeutics, which are usually designed to down-regulate protein expression, our technologies are designed to selectively up-regulate or down-regulate protein

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

PPMO, our next generation chemistry, features covalent attachment of a cell-penetrating peptide to a PMO with the goal of enhanced delivery into the cell. Based on our in-vivo preclinical research to date, we believe our proprietary class of PPMO compounds demonstrate an increase in dystrophin production and a more durable response compared to PMO. In addition, PPMO treatment in non-human primates is well tolerated and results in high levels of exon-skipping in skeletal, cardiac and smooth muscle tissues. Preclinical studies also indicate that PPMOs may require less frequent dosing than PMO, and that PPMOs could potentially be

tailored to reach other organs. We are targeting dosing the first patient with a PPMO candidate targeting exon 51 amenable children before the end of the 2017.

Our Clinical Programs

We are in the process of conducting, starting, or planning several studies in the U.S. and the EU for EXONDYS 51 and other product candidates designed to skip exons 45, 51 and 53 ("SRP-4045", "SRP-5051" and "SRP-4053", respectively). These are comprised of:

(i) studies we are currently conducting to further evaluate EXONDYS 51, including the Phase 3 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) additional EXONDYS 51 studies we are discussing with regulatory authorities and have initiated to comply with U.S. and/or EU regulatory requirements for the new drug applications ("NDA") and MAAs, respectively (e.g. a Phase 2 study on participants between the ages of six months and four years in connection with our Pediatric Investigation Plan in the EU);
(iii) studies we are planning to fulfill for our post-marketing FDA requirements/commitments for EXONDYS 51;
(iv) a randomized, double-blind dose-ranging study that we completed for SRP-4045 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 SRP-4053 for which Part I has been completed and has now transitioned into Part II, an open label efficacy and safety study; we are targeting a meeting with the FDA in the first quarter of 2018 to discuss SRP-4053;
(vi) ESSENCE, a placebo-controlled study with SRP-4045 and SRP-4053, which is enrolling patients in the U.S. and the EU, and for which we plan to have sites in Israel and Canada. We anticipate completing enrollment in ESSENCE by year-end 2017 or early in the first quarter of 2018;
(vii) additional Phase 1 studies we are planning to initiate for SRP-4053 and SRP-4045; and
(viii) a first in human, single ascending dose, study for SRP-5051 we are planning to initiate by year-end 2017.

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

Manufacturing

We believe we have developed proprietary state-of-the-art manufacturing and techniques that allow synthesis and purification of our product candidates to support both clinical development as well as commercialization. Our current main focus in manufacturing is to continue scaling up production of our PMO-based products and optimizing manufacturing for PPMO. We have entered into certain manufacturing and supply arrangements with third party suppliers which will in part utilize these techniques to support production of certain of our product candidates and their components. We have recently opened a facility in Andover, Massachusetts, which significantly enhances our research and development manufacturing capabilities. However, we currently do not have any of our own internal manufacturing capabilities to produce our product and product candidates for commercial and/or clinical use.

Cash, Cash Equivalents and Investments

As of September 30, 2017, we had approximately $618.4 million of cash, cash equivalents and investments, consisting of $617.6 million of cash and cash equivalents and $0.8 million restricted cash and investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve months.

The likelihood of our long-term success must be considered in light of the expenses, difficulties and delays frequently encountered in the development and commercialization of new pharmaceutical products, competitive factors in the marketplace, the

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

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations 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;
- inventory;
- research and development expense;
- stock-based compensation; and
- income taxes.

There have been no changes to our critical accounting policies and significant estimates as detailed in our Annual Report on Form 10-K for the year ended December 31, 2016.

Results of Operations for the Three and Nine Months Ended September 30, 2017 and 2016

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

	For the Three Months Ended			
	September 30,			
	2017	2016	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$45,954	$—	$45,954	NA
Total revenues	45,954	—	45,954	NA
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	3,078	—	3,078	NA
Research and development	34,239	34,349	(110)	(0)%
Selling, general and administrative	28,176	22,184	5,992	27 %
Settlement and license charges	25,588	—	25,588	NA
Amortization of in-licensed rights	780	—	780	NA

Total cost and expenses	91,861	56,533	35,328	62	%
Operating loss	(45,907)	(56,533)	10,626	(19	)%
Other income (loss):					
Interest income (expense) and other, net	184	(209)	393	(188	)%
Loss before income tax expense	(45,723)	(56,742)	11,019	(19	)%
Income tax expense	2,011	—	2,011	NA	
Net loss	$(47,734)	$(56,742)	$9,008	(16	)%
Net loss per share - basic and diluted	$(0.78)	$(1.18)	$0.40	(34	)%

	For the Nine Months Ended			
	September 30,			
	2017	2016	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$97,307	$—	$97,307	NA
Total revenues	97,307	—	97,307	NA
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	3,807	—	3,807	NA
Research and development	122,266	117,523	4,743	4 %
Selling, general and administrative	90,461	60,812	29,649	49 %
Settlement and license charges	28,427	—	28,427	NA
Amortization of in-licensed rights	837	—	837	NA
Total cost and expenses	245,798	178,335	67,463	38 %
Operating loss	(148,491)	(178,335)	29,844	(17)%
Other income (loss):				
Gain from sale of Priority Review Voucher	125,000	—	125,000	NA
Interest income (expense) and other, net	703	(478)	1,181	(247)%
Loss before income tax expense	(22,788)	(178,813)	156,025	(87)%
Income tax expense	3,902	—	3,902	NA
Net loss	$(26,690)	$(178,813)	$152,123	(85)%
Net loss per share - basic and diluted	$(0.47)	$(3.83)	$3.36	(88)%

Revenues

We record product revenues net of applicable discounts and allowances which include Medicaid rebates, Public Health Services chargebacks, prompt pay, co-pays and distribution and data fees. Reserves established for these discounts and allowances are classified as reductions of accounts receivable (if the amount is payable to our customer) or a liability (if the amount is payable to a party other than our customer). These reserves are based on estimates of the amounts earned or to be claimed on the related sales. Our estimates take into consideration current contractual and statutory requirements. Actual amounts may ultimately differ from our estimates. If actual results are different from our estimates, we adjust these estimates, which will have an effect on earnings in the period of adjustment. Product revenues, net for the three and nine months ended September 30, 2017 reflect sales from EXONDYS 51 in the U.S.

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

Our cost of sales (excluding amortization of in-licensed rights) relates to sales of EXONDYS 51 following its commercial launch in the U.S. Prior to receiving regulatory approval for EXONDYS 51 from the FDA in September 2016, we expensed such manufacturing and material costs as research and development expenses. Additionally, the cost of sales for the three and nine months ended September 30, 2017 also included approximately $2.3 million royalties to BioMarin as a result of a license agreement that was executed in July 2017.

For EXONDYS 51 sold during the three and nine months ended September 30, 2017, a majority of related manufacturing costs incurred had previously been expensed as research and development expenses, as such costs were incurred prior to the FDA approval of EXONDYS 51. Therefore, the cost of sales presented in the unaudited condensed consolidated statements of operations and comprehensive loss only included the cost of packaging and

labeling for commercial sales as well as royalty payments to BioMarin. If product related costs had not previously been expensed as research and development expenses prior to receiving FDA approval, the incremental cost to produce the EXONDYS 51 sold would have been approximately $2.5 million and $5.4 million for the three and nine months ended September 30, 2017, respectively.

Research and Development Expenses

Research and development expenses associated with our programs include clinical trial site costs, clinical manufacturing costs, costs incurred for consultants, up-front fees and milestones paid to third parties in connection with technologies which have not reached technological feasibility and do not have an alternative future use, and other external services, such as data management and

statistical analysis support, and materials and supplies used in support of clinical programs. Internal research and development expenses include salaries, stock-based compensation and allocation of our facility costs.

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

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

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

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

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

	For the Three Months Ended September 30,			
	2017	2016	Change $	Change %
	(in thousands)			
EXONDYS 51	$8,337	$17,966	$(9,629)	(54)%
Exon 45	5,279	2,544	2,735	108 %
Exon 53	4,569	1,837	2,732	149 %
Other projects	2,738	157	2,581	1,644 %
Internal research and development expenses	13,316	11,845	1,471	12 %
Total research and development expenses	$34,239	$34,349	$(110)	(0)%

	For the Nine Months Ended

	September 30, 2017	2016	Change $	Change %
	(in thousands)			
EXONDYS 51	$27,821	$57,337	$(29,516)	(51)%
Exon 45	13,266	4,302	8,964	208 %
Exon 53	12,709	7,584	5,125	68 %
Other projects	7,491	1,222	6,269	513 %
Summit and UWA collaboration and license expenses	22,000	7,000	15,000	214 %
Internal research and development expenses	38,979	40,078	(1,099)	(3)%
Total research and development expenses	$122,266	$117,523	$4,743	4 %

The Company has revised the presentation as well as the certain caption in the research and development expenses by project tables presented above. “Summit and UWA collaboration and license expenses” of $7.0 million for the nine months ended September 30, 2016 was reclassified out of EXONDYS 51 and presented separately in the table to conform to current year presentation.

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

	For the Three Months Ended September 30,			
	2017	2016	Change $	Change %
	(in thousands)			
Clinical and manufacturing expenses	16,381	20,773	(4,392)	(21)%
Compensation and other personnel expenses	5,957	5,477	480	9 %
Preclinical expenses	3,304	612	2,692	440 %
Professional services	2,860	1,718	1,142	66 %
Facility-related expenses	2,269	1,645	624	38 %
Stock-based compensation	1,812	2,674	(862)	(32)%
Research and other	1,656	1,450	206	14 %
Total research and development expenses	$34,239	$34,349	$(110)	(0)%

	For the Nine Months Ended September 30,			
	2017	2016	Change $	Change %
	(in thousands)			
Clinical and manufacturing expenses	$50,650	$65,681	$(15,031)	(23)%
Summit and UWA collaboration and license expenses	22,000	7,000	15,000	214 %
Compensation and other personnel expenses	17,738	18,116	(378)	(2)%
Preclinical expenses	7,326	2,583	4,743	184 %
Professional services	7,327	5,757	1,570	27 %
Facility-related expenses	6,636	5,736	900	16 %
Stock-based compensation	5,881	7,527	(1,646)	(22)%
Research and other	4,708	5,123	(415)	(8)%
Total research and development expenses	$122,266	$117,523	$4,743	4 %

Research and development expenses for the three months ended September 30, 2017 was flat compared with the three months ended September 30, 2016. There were increases of $2.7 million in preclinical expenses due to a ramp-up of preclinical studies in our PPMO platform and other follow-on exons, $1.1 million in professional services, $0.6 million in facility-related expenses and $0.5 million in compensation and other personnel expenses which was primarily driven by increased headcount. These increases were offset by decreases of $4.4 million in clinical and manufacturing expenses due to lower manufacturing expenses because of the capitalization of inventory following the approval of EXONDYS 51 by the FDA partially offset by increased patient enrollment in our ongoing clinical trials as well as $0.9 million in stock-based compensation. In September 2017, one of the performance milestones related to the restricted stock units granted to executives in March 2017 became probable of being achieved within the required

timeline and, accordingly, we recognized approximately $0.1 million in stock-based compensation. In September 2016, two performance milestones for stock options with performance conditions granted in June 2013 and February 2016 were achieved as a result of the regulatory approval of EXONDYS 51 by the FDA and, accordingly, we recognized approximately $0.8 million in stock-based compensation.

Research and development expenses for the nine months ended September 30, 2017 increased by $4.7 million, or 4%, compared with the nine months ended September 30, 2016. This was primarily driven by a milestone payment of $22.0 million to Summit as the milestone of the last patient dosed in the safety arm cohort to the PhaseOut DMD study was achieved in May 2017 and increases of $4.7 million in preclinical expenses due to a ramp-up of preclinical studies in our PPMO platform and other follow-on exons, $1.6 million in professional services, and $0.9 million in facility-related expenses due to increased headcount. The increases were partially offset by decreases of $15.0 million in clinical and manufacturing expenses due to lower manufacturing expenses because of the capitalization of inventory following the approval of EXONDYS 51 by the FDA partially offset by increased patient enrollment in our ongoing clinical trials and $1.6 million in stock-based compensation.

Selling, General and Administrative Expenses

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

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

	For the Three Months Ended September 30,			
	2017	2016	Change $	Change %
	(in thousands)			
Professional services	$10,713	4,283	$6,430	150 %
Compensation and other personnel expenses	9,204	8,238	966	12 %
Stock-based compensation	5,178	6,899	(1,721)	(25)%
Former CEO severance	137	—	137	NA
Facility-related expenses	1,203	1,291	(88)	(7)%
Other	1,741	1,473	268	18 %
Total selling, general and administrative expenses	$28,176	$22,184	$5,992	27 %

	For the Nine Months Ended September 30,			
	2017	2016	Change $	Change %
	(in thousands)			
Professional services	$31,642	$13,894	$17,748	128 %
Compensation and other personnel expenses	26,718	22,308	4,410	20 %
Stock-based compensation	15,087	15,566	(479)	(3)%
Former CEO severance	3,537	—	3,537	NA
Facility-related expenses	4,355	3,385	970	29 %
Restructuring expenses	2,589	639	1,950	305 %
Other	6,533	5,020	1,513	30 %
Total selling, general and administrative expenses	$90,461	$60,812	$29,649	49 %

The Company has revised the presentation as well as the certain caption in the selling, general and administrative expenses tables presented above. For the nine months ended September 30, 2016, “restructuring expenses” of $0.6 million were reclassified out of “compensation and other personnel expenses” and presented separately in the table to conform to current year presentation.

Selling, general and administrative expenses for the three months ended September 30, 2017 increased by $6.0 million, or 27%, compared with the three months ended September 30, 2016. This was primarily driven by increases of $6.4 million in professional services primarily due to increased legal fees because of on-going litigations and global

commercial expansion and $1.0 million in compensation and other personnel expenses primarily due to increase in headcount. The increases were partially offset by a decrease of $1.7 million in stock-based compensation. In September 2017, one of the performance milestones related to the restricted stock units granted to executives in March 2017 became probable of being achieved within the required timeline and, accordingly, we recognized approximately $0.2 million in stock-based compensation. In September 2016, two performance milestones for stock options with performance conditions granted in June 2013 and February 2016 were achieved as a result of the regulatory approval of EXONDYS 51 by the FDA and, accordingly, we recognized approximately $2.7 million in stock-based compensation.

Selling, general and administrative expenses for the nine months ended September 30, 2017 increased by $29.6 million, or 49%, compared with the nine months ended September 30, 2016. This was primarily driven by increases of $17.7 million in professional services primarily due to increased legal fees because of on-going litigations and global commercial expansion, $4.4 million in compensation and other personnel expense due to increased headcount, $3.5 million in estimated severance due to the resignation of our former CEO, $2.0 million in restructuring expenses related to the closure of our Corvallis, Oregon site and $1.0 million facility-related expenses. The increases were partially offset by $0.5 million in stock-based compensation.

Settlement and License Charges

In July 2017, we and BioMarin Leiden Holding BV, BioMarin Nederlands BV and BioMarin Technologies BV (collectively, the "BioMarin Parties") executed a license agreement (the "License Agreement"), pursuant to which the BioMarin Parties granted us a royalty-bearing, worldwide license under patent rights ("Licensed Patents") and know-how ("Licensed Know-How") controlled by the BioMarin Parties with respect to the BioMarin Parties' DMD program, which are potentially necessary or useful for the treatment of DMD, to practice and exploit the Licensed Patents and Licensed Know-How in all fields of use and for all purposes, including to develop and commercialize antisense oligonucleotide products that target one or more exons of the dystrophin gene to induce exon skipping, including eteplirsen. In addition, in July 2017, we and The University of Western Australia ("UWA") on the one hand, and the BioMarin Parties and Academisch Ziekenhuis Leiden ("AZL") on the other hand (collectively, the "Settlement Parties"), executed a settlement agreement (the "Settlement Agreement") pursuant to which all legal actions in the U.S. and certain legal actions in Europe would be stopped or withdrawn as between the Settlement Parties. Under the terms of the License Agreement and the Settlement Agreement, we agreed to make total up-front payments of $35.0 million upon execution of these agreements, consisting of $20.0 million under the Settlement Agreement and $15.0 million under the License Agreement. Additionally, we may be liable for up to approximately $65.0 million in regulatory and sales milestones for eteplirsen as well as exon 45 and exon 53 skipping product candidates. The BioMarin Parties will also be eligible to receive royalty payments, ranging from 4% - 8%, which will expire in December 2023 in the U.S. and September 2024 in the EU. For the three and nine months ended September 30, 2017, we recognized settlement and license charges of $25.6 million and $28.4 million, respectively.

Amortization of In-licensed Rights

Amortization of in-license rights relate to the two agreements we entered into with BioMarin and UWA in July 2017 and April 2011, respectively. We recorded an in-licensed right asset of approximately $6.6 million as a result of the settlement agreement with BioMarin. Additionally, following the first sale of EXONDYS 51 in September 2016, we recorded an in-licensed right asset of $1.0 million related to a license agreement with UWA. Both in-licensed rights are being amortized on a straight-line basis over the life of the patent from the first commercial sale of EXONDYS 51. For both the three and nine months ended September 30, 2017, we recorded amortization of in-licensed rights of approximately $0.8 million.

Gain from Sale of Priority Review Voucher

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

Interest income (expense) and other, net

Interest income (expense) and other, net, primarily consists of interest income on our cash, cash equivalents and investments, interest expense and rental income and loss. Our cash equivalents and investments consist of commercial paper, government and government agency debt securities, money market investments and certificates of deposit. Interest expense includes interest accrued on our term loans, revolving line of credit and mortgage loans related to our Corvallis, Oregon property. Rental income is from leasing excess space in some of our facilities.

For the three and nine months ended September 30, 2017, interest income and other, net was approximately $0.2 million and $0.7 million, respectively. For the three and nine months ended September 30, 2016, interest expense and

other, net was approximately $0.2 million and $0.5 million, respectively. The favorable changes for both periods primarily reflected increased interest income from higher balances of cash, cash equivalents and investments.

Income tax expense

Primarily corresponding to the gain from sale of the PRV, income tax expense for the three and nine months ended September 30, 2017 was approximately $2.0 million and $3.9 million, respectively, related to alternative minimum tax. Income tax expense for the same period in 2016 was zero as we were in a loss position.

Liquidity and Capital Resources

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

	As of September 30, 2017	As of December 31, 2016	Change $	Change %
	(in thousands)			
Financial assets:				
Cash and cash equivalents	$617,630	$122,420	$495,210	405 %
Short-term investments	—	195,425	(195,425)	(100)%
Restricted cash and investments	784	11,479	(10,695)	(93)%
Total cash, cash equivalents and investments	$618,414	$329,324	$289,090	88 %
Borrowings:				
Current portion of long-term debt	$4,732	$10,108	$(5,376)	(53)%
Long-term debt	26,550	6,042	20,508	339 %
Total borrowings	$31,282	$16,150	$15,132	94 %
Working capital				
Current assets	$735,608	$373,476	$362,132	97 %
Current liabilities	70,470	75,422	(4,952)	(7)%
Total working capital	$665,138	$298,054	$367,084	123 %

For the period ended September 30, 2017, our principal source of liquidity was derived from proceeds from the sale of the PRV, equity and debt financings and product sales of EXONDYS 51. For the period ended December 31, 2016, our principal source of liquidity was from equity financings and product sales. Our principal uses of cash are research and development expenses, selling, general and administrative expenses, investments, capital expenditures and other working capital requirements.

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

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

Our cash requirements are expected to continue to increase as we advance our research, development and commercialization programs and we expect to seek additional financings primarily from, but not limited to, the sale and issuance of equity, debt securities or the licensing or sale of our technologies. We cannot provide assurances that

financing will be available when and as needed or that, if available, the financings will be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we require, this would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

Cash Flows

	For the Nine Months Ended September 30,			
	2017	2016	Change $	Change %
	(in thousands)			
Cash provided by (used in)				
Operating activities	$(200,938)	$(164,587)	$(36,351)	22 %
Investing activities	314,880	108,581	206,299	190 %
Financing activities	381,269	370,842	10,427	3 %
Increase in cash and cash equivalents	$495,211	$314,836	$180,375	57 %

Operating Activities. Cash used in operating activities increased by $36.4 million for the nine months ended September 30, 2017 compared with the nine months ended September 30, 2016. This was primarily due to unfavorable changes of $65.1 million in operating assets and liabilities primarily related to increases in accounts receivables and inventory as we launched EXONDYS 51 partially offset by an increase of $1.7 million in non-cash adjustments and a decrease of $27.1 million in net loss excluding the gain from sale of PRV driven by product sales for EXONDYS 51 partially offset by increases in research and development expenses and selling, general and administrative expenses.

Investing Activities. The cash provided by investing activities increased by $206.3 million for the nine months ended September 30, 2017 compared with the nine months ended September 30, 2016. This was driven by proceeds of $125.0 million from sale of the PRV and increases of $184.1 million from the maturity/sale of available-for-sale securities and $10.7 million from the maturity of a restricted investment partially offset by increases of purchase of available-for-sales securities of $100.3 million, $5.7 million in purchases of property and equipment and $7.5 million in purchases of intangible assets.

Financing Activities. Cash provided by financing activities increased by $10.4 million for the nine months ended September 30, 2017 compared with the nine months ended September 30, 2016. This was primarily driven by increases of $20.6 million in proceeds from borrowings, net of payments on debt obligations and certain debt issuance costs, and $0.8 million in proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program. The increases were partially offset by a decrease of $11.0 million in proceeds from sales of common stock.

Milestone Obligations

As of September 30, 2017, we were obligated to make up to $808.5 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 and sales milestones. As of September 30, 2017, we made an up-front cash payment of $35.0 million to BioMarin related to the license and settlement agreements. Accordingly, we recorded an intangible asset in the U.S. of $6.6 million on our unaudited condensed consolidated balance sheet. For the three and nine months ended September 30, 2017, we recorded settlement and license charges of $25.6 million and $28.4 million, respectively, on our unaudited condensed consolidated statements of operations and comprehensive loss. Additionally, for the nine months ended September 30, 2017 and 2016, we recognized $22.0 million and $7.0 million milestone and up-front payments to Summit and UWA, respectively, as research and development expense.

Other Funding Commitments

As of September 30, 2017, we have several on-going clinical studies in various clinical trial stages. Our most significant clinical trial expenditures are to contract research organizations (“CROs”). The CRO contracts are generally cancellable at our option. As of September 30, 2017, we have approximately $54.4 million in cancellable future commitments based on existing CRO contracts.

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, 2017.

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, 2017, we had approximately $618.4 million of cash, cash equivalents and investments, comprised of $617.6 million of cash and cash equivalents and $0.8 million restricted cash and investments. Our cash equivalents consist of commercial paper, government and government agency debt securities, money market investments and certificates of deposit. The fair value of cash equivalents is subject to change as a result of potential changes in market interest rates. The potential change in fair value for interest rate sensitive instruments has been assessed on a hypothetical 10 basis point adverse movement across all maturities. As of September 30, 2017, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of less than $0.1 million to our interest rate sensitive instruments.

Item 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, 2017, under the supervision and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of our disclosure controls and procedures pursuant to paragraph (b) of Rules 13a-15 and 15d-15 under the Securities Exchange Act of 1934 (the “Exchange Act”). The purpose of this evaluation was to determine whether as of the evaluation date our disclosure controls and procedures were effective to provide reasonable assurance that the information we are required to disclose in our filings with the SEC under the Exchange Act (i) is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms and (ii) is accumulated and communicated to our management, including our Chief Executive Officer and our Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of September 30, 2017, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarterly period ended September 30, 2017, 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 18, 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, 2016, are descriptions of risks and uncertainties that could cause actual results to differ materially from the results contemplated by the forward-looking statements contained in this report. Because of the following factors, as well as other variables affecting our operating results, past financial performance should not be considered a reliable indicator of future performance and investors should not use historical trends to anticipate results or trends in future periods. The risks and uncertainties described below are not the only ones facing us. Other events that we do not currently anticipate or that we currently deem immaterial also affect our results of operations and financial condition.

Risks Related to Our Business

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

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

- the effectiveness of our sales, managed markets, marketing efforts and support for EXONDYS 51;
- the consistency of any new data we collect and analyses we conduct with prior results, whether they support a favorable safety and efficacy profile of EXONDYS 51 and any potential impact on our FDA accelerated approval status and/or FDA package insert for EXONDYS 51;
- the effectiveness of our ongoing EXONDYS 51 commercialization activities, including negotiating and entering into any additional commercial, supply and distribution contracts, scaling up manufacturing and hiring any additional personnel as needed to support commercial efforts;
- our ability to comply with FDA post-marketing requirements and commitments, including through successfully conducting additional studies that confirm clinical efficacy and safety of EXONDYS 51 and acceptance of the same by the FDA and medical community since continued approval for this indication may be contingent upon verification of the clinical benefit in confirmatory trials;
- the occurrence of any side effects, adverse reactions or misuse, or any unfavorable publicity in these areas;
- the cost-effectiveness of EXONDYS 51 and whether we can consistently manufacture it in commercial quantities and at acceptable costs;
- the rate and consistency with which EXONDYS 51 is prescribed by physicians, which depends on physicians' views on the safety and efficacy of EXONDYS 51;

- our ability to secure and maintain adequate reimbursement for EXONDYS 51, including during re-authorizations processes that may be required for patients who initially obtained coverage by third parties, including government payors, managed care organizations and private health insurers;
- our ability to obtain and maintain patent protection for EXONDYS 51, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing on the proprietary rights of third parties;
- the development or commercialization of competing products or therapies for the treatment of DMD, or its symptoms;
- our ability to increase awareness of the importance of genetic testing and knowing/understanding DMD mutations, and identifying and addressing procedural barriers to obtaining therapy;
- our ability to remain compliant with laws and regulations that apply to us and our commercial activities;

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

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

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

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

Although we contracted with third party distributors to distribute eteplirsen in certain countries outside the U.S. on a named patient basis, and initiated a limited launch of an ex-U.S. eteplirsen MAP, which we plan to expand to other jurisdictions in the future, and although we continue to pursue regulatory approval of eteplirsen in certain targeted jurisdictions, such as the EU and Israel, we may not be successful in expanding access to eteplirsen nor produce any significant revenues from eteplirsen sales outside of the U.S. for various reasons. For example, healthcare providers in MAP jurisdictions may not be convinced that their patients can benefit from eteplirsen or may prefer to wait until such time as eteplirsen is approved by a regulatory authority in their country before prescribing eteplirsen. Even if a healthcare provider is interested in obtaining access to eteplirsen for its patient through the MAP, the patient will not be able to obtain access to eteplirsen if payment for the drug is not secured, which may be difficult due to the cost of eteplirsen. Additionally, we may not be able to obtain regulatory approval in the jurisdictions we have targeted such as the EU if our product approval applications and data packages submitted to regulatory authorities and any additional data and analyses we submit in response to requests and concerns from regulatory authorities do not support or convince regulatory authorities of the safety and efficacy of eteplirsen. If we fail to obtain regulatory approvals, particularly for our eteplirsen MAA in the EU, our ability to make revenues from eteplirsen sales outside of the U.S. will be extremely limited. Even if we are successful in obtaining regulatory approval of eteplirsen outside of the U.S., our revenue earning capacity will depend on commercial and medical infrastructure, pricing and reimbursement negotiations and decisions with third party payors, including government payors. See "— Even though EXONDYS 51 has been approved for marketing in the U.S., we may never receive approval to commercialize EXONDYS 51 outside of the U.S."

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

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

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

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

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

We currently do not have the internal ability to undertake the manufacturing process for EXONDYS 51 or our product candidates in the quantities needed to meet commercial, clinical or MAP demand for EXONDYS 51, or to conduct our research and development programs and conduct clinical trials for our product candidates, including PPMO. Therefore, we rely on, and expect to continue relying on for the foreseeable future, a limited number of third parties to manufacture and supply materials (including raw materials and subunits), API and drug product, as well as to perform additional steps in the manufacturing process, such as labeling and packaging of vials and storage of EXONDYS 51 and our product candidates. There are a limited number of third parties with facilities and capabilities suited for the manufacturing process of EXONDYS 51 and our product candidates, which creates a heightened risk that we may not be able to obtain materials and APIs in the quantity and purity that we require. In addition, the process for adding new manufacturing capacity can be lengthy and could cause delays in our development efforts. Any interruption of the development or operation of those facilities due to, among other reasons, events such as order delays for equipment or materials, equipment malfunction, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability, production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters such as earthquake or fire, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available EXONDYS 51, product candidates or materials.

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

We have, through our third party manufacturers, produced or are in the process of producing supply of our product candidates and EXONDYS 51, respectively, based on our current understanding of market demands and our anticipated needs for our research and development efforts, clinical trials, MAPs and commercial sales. In light of the limited number of third parties with the expertise to produce EXONDYS 51 and our product candidates, the lead time needed to manufacture them, and the availability of underlying materials, we may not be able to, in a timely manner or at all, establish or maintain sufficient commercial and other manufacturing arrangements on the commercially reasonable terms necessary to provide adequate supply of EXONDYS 51 and our other product candidates to meet demands that meet or exceed our projected needs. Furthermore, we may not be able to obtain the significant financial capital that may be required in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for EXONDYS 51 and our product candidates, such parties may not comply with the terms and timelines they have agreed to for various reasons, some of which may be out of their or our control, which could impact our ability to execute our business plans on expected or required timelines in connection with the commercialization of EXONDYS 51 and the continued development of our product candidates, including our follow-on exon-skipping product candidates and PPMO. 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, EMA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations. While we work diligently with all contract manufacturers to maintain full compliance, we do not have direct control over a third party manufacturer's compliance with these regulations and requirements. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of EXONDYS 51 and our product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively. The failure to achieve and maintain compliance with cGMP and other applicable government regulations, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in product recalls, clinical holds, delayed or withheld approvals, patient injury or death. This risk is particularly heightened as we optimize manufacturing for follow-on exon skipping products and next-generation technologies such as PPMO. If our contract manufacturers fail to adhere to applicable cGMP and other applicable government regulations, or experience manufacturing problems, we will suffer significant consequences, including product seizures or recalls, postponement or cancellation

of clinical trials, loss or delay of product approval, fines and sanctions, loss of revenue, termination of the development of a product candidate, reputational damage, shipment delays, inventory shortages, inventory write-offs and other product-related charges and increased manufacturing costs. If we experience any of these results, the success of our commercialization of EXONDYS 51 and/or our development efforts for our product candidates, including SRP-4053, SRP-5051 and SRP-4045, could be significantly delayed, fail or otherwise be negatively impacted.

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

We are working to increase manufacturing capacity and scale up production of some of the components of our drug products. During the remainder of 2017, our focus remains on (i) achieving larger-scale manufacturing capacity for EXONDYS 51 throughout the manufacturing supply chain (ii) continuing to increase material and API production capacity to provide the anticipated amounts of drug product needed for our planned studies for our product candidates and (iii) optimizing manufacturing for our follow-on exon skipping product candidates, including PPMO. We may not be able to successfully increase manufacturing capacity or scale up the production of materials, APIs and drug products, whether in collaboration with third party manufacturers or on our own, in a manner that is safe, compliant with cGMP conditions or other applicable legal or regulatory requirements, in a cost-effective manner, in a time frame required to meet our timeline for commercialization, clinical trials and other business plans, or at all. Compliance with cGMP requirements and other quality issues may arise during our efforts to increase manufacturing capacity and scale up production with our current or any new contract manufacturers. These issues may arise in connection with the underlying materials, the inherent properties of EXONDYS 51 or a product candidate, EXONDYS 51 or a product candidate in combination with other components added during the manufacturing and packaging process or during shipping and storage of the APIs or finished drug product. In addition, in order to release EXONDYS 51 for commercial use and demonstrate stability of product candidates for use in clinical trials (and any subsequent drug products for commercial use), our manufacturing processes and analytical methods must be validated in accordance with regulatory guidelines. We may not be able to successfully validate, or maintain validation of, our manufacturing processes and analytical methods or demonstrate adequate purity, stability or comparability of EXONDYS 51 or our product candidates in a timely or cost-effective manner, or at all. If we are unable to successfully validate our manufacturing processes and analytical methods or to demonstrate adequate purity, stability or comparability, the commercial availability of EXONDYS 51 and the continued development and/or regulatory approval of our product candidates, including PPMO, may be delayed or otherwise negatively impacted, which could significantly harm our business.

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

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

We rely on third parties to commercially distribute EXONDYS 51 to patients in the U.S. We have contracted with a third party logistics company to warehouse EXONDYS 51 and with distributors and specialty pharmacies to sell and

distribute it to patients. A specialty pharmacy is a pharmacy that specializes in the dispensing of medications for complex or chronic conditions that require a high level of patient education and ongoing management.

This distribution network requires significant coordination with our sales and marketing and finance organizations. In addition, failure to coordinate financial systems could negatively impact our ability to accurately report product revenue from EXONDYS 51. If we are unable to effectively manage the distribution process, the sales of EXONDYS 51, as well as any future products we may commercialize, could be delayed or severely compromised and our results of operations may be harmed.

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

- not provide us with accurate or timely information regarding their inventories, the number of patients who are using EXONDYS 51 or serious adverse events and/or product complaints regarding EXONDYS 51;
- not effectively sell or support EXONDYS 51;
- reduce or discontinue their efforts to sell or support EXONDYS 51;
- not devote the resources necessary to sell EXONDYS 51 in the volumes and within the time frame we expect;
- be unable to satisfy financial obligations to us or others; or
- cease operations.

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

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

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

We have hired and trained a commercial team and put in the organizational infrastructure we believe we need to support the commercial success of EXONDYS 51 in the U.S. Factors that may inhibit our efforts to maintain and further develop commercial capabilities include:

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

If we are not successful in maintaining an effective commercial, sales and marketing infrastructure, we will encounter difficulty in achieving, maintaining or increasing projected sales of EXONDYS 51 in the U.S., which would adversely affect our business and financial condition.

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

Our ability to successfully maintain and/or increase EXONDYS 51 sales in the U.S. depends in part on the coverage and reimbursement levels set by governmental authorities, private health insurers and other third party payors. Third party payors are increasingly challenging the effectiveness of and prices charged for medical products and services. We may not be able to obtain or maintain adequate third party coverage or reimbursement for EXONDYS 51, or we may be required to sell EXONDYS 51 at an unsatisfactory price.

We expect that private insurers will continue to consider the efficacy, cost-effectiveness and safety of EXONDYS 51, including any new data and analyses that we are able to collect and make available in a compliant manner, in determining whether to approve reimbursement for EXONDYS 51 and at what levels. If any new data and information we collect is not favorable, third party insurers may make coverage decisions that negatively impact sales of EXONDYS 51. We continue to have discussions with payors,

some of which may eventually deny coverage. We may not receive approval for reimbursement of EXONDYS 51 from additional private insurers on a satisfactory rate or basis, in which case our business would be materially adversely affected. In addition, obtaining these approvals can be a time consuming and expensive process. Our business would be materially adversely affected if we are not able to maintain favorable coverage decisions and/or fail to receive additional favorable coverage decisions from third party insurers, in particular during re-authorization processes for patients that have already initiated therapy. Our business could also be adversely affected if private insurers, including managed care organizations, the Medicare or Medicaid programs or other reimbursing bodies or payors limit the indications for which EXONDYS 51 will be reimbursed.

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

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

We expect to experience pricing pressures in connection with the sale of EXONDYS 51 and our future products due to a number of factors, including current and future healthcare reforms and initiatives by government health programs and private insurers (including managed care plans) to reduce healthcare costs.

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

The U.S. government and individual states have aggressively pursued healthcare reform, as evidenced by the passing of the Affordable Care Act and the current debate concerning modifications to or repeal of such Act. The Affordable Care Act substantially changed the way healthcare is financed by both governmental and private insurers and contains a number of provisions that affect coverage and reimbursement of drug products and/or that could potentially reduce the demand for pharmaceutical products such as increasing drug rebates under state Medicaid programs for brand name prescription drugs and extending those rebates to Medicaid managed care and assessing a fee on manufacturers and importers of brand name prescription drugs reimbursed under certain government programs, including Medicare and Medicaid. Other aspects of healthcare reform, such as expanded government enforcement authority and heightened standards that could increase compliance-related costs, could also affect our business. Modifications to or repeal of all or certain provisions of the Affordable Care Act are being actively debated as a result of the outcome of the 2016 presidential election and Republicans maintaining control of both houses of Congress, consistent with statements made by President Donald Trump and members of Congress both during the presidential campaign and continuing into 2017. We cannot predict the ultimate content, timing or effect of any changes to the Affordable Care Act or other federal and state reform efforts. There is no assurance that federal or state health care reform will not adversely affect our future business and financial results, and we cannot predict how future federal or state legislative, judicial or administrative changes relating to healthcare reform will affect our business.

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription

drugs. We anticipate that the U.S. Congress, state legislatures and the private sector will continue to consider and may adopt healthcare policies intended to curb rising healthcare costs. These cost containment measures may include:

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

- expansion of use of managed care systems in which healthcare providers contract to provide comprehensive healthcare for a fixed cost per person; and
- prohibition on direct-to-consumer advertising or drug marketing practices.

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

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

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

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

Continued approval for this indication is contingent upon completing various post-marketing requirements and commitments, including the requirement to conduct a randomized, controlled clinical trial to verify the drug's clinical benefit. These post-approval requirements and commitments may not be feasible and/or could impose significant burdens and costs on us; could negatively impact our development, manufacturing and supply of EXONDYS 51; and could negatively impact our financial results. Failure to meet post-approval commitments and requirements, including completion of enrollment and in particular, any failure to obtain positive safety and efficacy data from our ongoing and planned EXONDYS 51 studies, would lead to negative regulatory action from the FDA and/or withdrawal of regulatory approval of EXONDYS 51, and could also negatively impact a decision from EMA on our MAA. In addition, if additional data we collect on eteplirsen in connection with our MAA does not support the safety and efficacy or EXONDYS 51, our approval status in the U.S. could be negatively impacted.

Manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP regulations. Drug product manufacturers are required to continuously monitor and report adverse events from clinical trials and commercial use of the product. If we or a regulatory agency discover previously unknown adverse events or events of unanticipated severity or frequency, a regulatory agency may require labeling changes implementation of risk evaluation and mitigation strategy program, or additional post-marketing studies or clinical trials. If we or a regulatory agency discover previously unknown problems with a product, such as problems with a facility where the API or drug product is manufactured or tested, a regulatory agency may impose restrictions on that product and/or the manufacturer, including removal of specific product lots from the market, withdrawal of the product from the market, or suspension of manufacturing. Sponsors of drugs approved under FDA accelerated approval provisions also are required to submit to FDA, at least 30 days before initial use, all promotional materials intended for use after the first 120 days following marketing approval. If we or the manufacturing facilities for EXONDYS 51 fail to comply with applicable regulatory

requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw or alter the conditions of our marketing approval;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- suspend any ongoing clinical trials;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- refuse to approve pending applications or supplements to applications submitted by us;

- suspend or impose restrictions on operations, including costly new manufacturing requirements;
- seize or detain products, refuse to permit the import or export of products or require us to initiate a product recall; or
- refuse to allow us to enter into supply contracts, including government contracts.

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

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

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

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

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

- we may not be able to validate our manufacturing process to the satisfaction of regulatory authorities outside the U.S. or demonstrate adequate cGMP compliance; or
- regulatory authorities outside the U.S. may adopt new or revised approval policies and regulations.

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

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

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

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

- our ability to demonstrate to the medical community, including specialists who may purchase or prescribe EXONDYS 51, the clinical efficacy and safety of EXONDYS 51 as the prescription product of choice DMD amenable to exon-51 skipping in the U.S.;
- the effectiveness of our sales and marketing organizations and distribution networks;
- the ability of patients or providers to be adequately reimbursed for EXONDYS 51 in a timely manner from government and private payors;
- the actual and perceived efficacy and safety profile of EXONDYS 51, particularly if unanticipated adverse events related to EXONDYS 51 treatment arise and create safety concerns among potential patients or prescribers or if new data and analyses we obtain for eteplirsen do not support, or are interpreted by some parties to not support, the efficacy of EXONDYS 51; and
- the efficacy and safety of our other exon-skipping product candidates, including our exon 45 and exon 53 product candidates, and third parties' competitive therapies.

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

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

our product development efforts and our sales, marketing and manufacturing expenses.

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

To date, we have been granted orphan drug exclusivity for EXONDYS 51 in the U.S and an orphan drug designation in the EU for eteplirsen. Product candidates granted orphan status in Europe can be provided with up to ten years of marketing exclusivity, meaning that another application for marketing authorization of a later, similar medicinal product for the same therapeutic indication

will generally not be approved in Europe during that time period. Although we may have product candidates that obtain orphan drug exclusivity in Europe, the orphan status and associated exclusivity period may be modified for several reasons, including a significant change to the orphan medicinal product designations or status criteria after-market authorization of the orphan product (e.g., product profitability exceeds the criteria for orphan drug designation), problems with the production or supply of the orphan drug, or a competitor drug, although similar, is safer, more effective or otherwise clinically superior than the initial orphan drug.

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

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

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

Physicians are permitted to prescribe drug products for uses that are not described in the product’s labeling and that differ from those approved by applicable regulatory agencies. Off-label uses are common across medical specialties. Although the FDA and other regulatory agencies do not regulate a physician’s choice of treatments, the FDA and other regulatory agencies do generally prohibit advertising and promotion of off-label uses of approved drug products or promotion of an approved drug on information that is not in the final, FDA-approved label for a product and restrict communications on off-label use. Accordingly, we may not promote EXONDYS 51 in the U.S. for use in any

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

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

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

Other than EXONDYS 51, which the FDA approved for use in the U.S. in September 2016 and for which we filed an MAA in November 2016 with the EMA, our most advanced product candidates are exon 45 and 53 skipping products. 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/commitments to verify and describe clinical benefit. The exon 53-skipping product candidate, which we are working on with the SKIP-NMD consortium, is currently in the clinic in EU. The Part I dose-titration portion of this Phase 1/2a study has been completed and Part II open label portion of the study is ongoing. We have also completed the dose titration portion and are conducting the open-label portion of a study for our exon 45-skipping product candidate. Additionally, we are enrolling patients in the U.S. and EU, and working towards initiating sites in Israel and Canada for a clinical trial using exon 45- and 53-skipping product candidates, which we refer to as the ESSENCE study. We also plan to initiate a first in human study for PPMO ("SRP-5051") DMD exon 51 by year-end 2017. 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 and the exon 53-skipping product candidate we are developing with the SKIP-NMD consortium, each for DMD, are in active clinical development. Our other product candidates, including our anti-bacterials and AVI-7537 in Ebola and AVI-7288, are in discovery, pre-clinical development or inactive. Given the FDA approval of EXONDYS 51, we expect that much of our effort and many of our expenditures over the next several years will be devoted to clinical development and regulatory activities associated with EXONDYS 51 and other exon-skipping candidates as part of our larger follow-on exon strategy in DMD, our other disease candidates, our proprietary chemistry, and other potential therapeutic areas that provide long-term market opportunities. We may be delayed, restricted, or unable to further develop our active and other product candidates or successfully obtain approvals needed to market them. Although EXONDYS 51 was approved under accelerated approval by the FDA in the U.S., we may not be able to obtain an approval of EXONDYS 51 in the EU.

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

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

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

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive pre-clinical and clinical studies that the product candidate is safe and effective in humans. Ongoing

and future pre-clinical and clinical trials of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. For example, although the pre-clinical data for PPMO collected to date is promising, the additional data we collect, including in the clinic, may not be consistent with the pre-clinical data or show a safe benefit that warrants further development or pursuit of a regulatory approval for PPMO product candidates. Furthermore, success in pre-clinical and early clinical trials does not ensure that the subsequent trials will be successful, nor does it predict final results of a confirmatory trial. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, including for those that are based on our PMO-based technologies, then the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld or withdrawn. For example, we cannot provide assurances that data from our EXONDYS 51 ongoing studies will be positive and consistent through the study periods or that the interpretation by regulators, such as the FDA or EMA, of the data we collect for our product candidates will be consistent with our interpretations.

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

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

- Our non-clinical, clinical, Chemistry, Manufacturing and Controls and other data and analyses from past, current and future studies for any of our product candidates may not be sufficient to meet regulatory requirements for marketing application approvals. The regulatory authorities could disagree with our interpretations and conclusions regarding data we provide in connection with NDA or MAA submissions for one or more of our product candidates, and may delay, reject or refuse to accept for review, or approve any NDA or MAA submission we make or identify additional requirements for product approval to be submitted upon completion, if ever. In addition, in the U.S., an FDA advisory committee could determine that our data are insufficient to provide a positive recommendation for approval of any NDA we submit to the FDA. Even if we meet FDA requirements and an advisory committee votes to recommend approval of an NDA submission, the FDA could still disagree with the advisory committee's recommendation and deny approval of a product candidate based on their review.
- The regulatory approval process for product candidates targeting orphan diseases, such as DMD, that use new technologies and processes, such as antisense oligonucleotide therapies, and alternative approaches or endpoints for the determination of efficacy is uncertain due to, among other factors, evolving interpretations of a new therapeutic class, the broad discretion of regulatory authorities, lack of precedent, varying levels of applicable expertise of regulators or their advisory committees, scientific developments, changes in the competitor landscape, shifting political priorities and changes in applicable laws, rules or regulations and interpretations of the same. We cannot be sure that any of our product candidates will qualify for accelerated approval or any other expedited development, review and approval programs, or that, if a drug does qualify, that the product candidates will be approved, will be accepted as part of any such program or that the review time will be shorter than a standard review. As a result of uncertainty in the approval process for products intended to treat serious rare diseases, we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned NDAs and MAAs for our product candidates, in a timely manner, or at all. Examples of such requests or requirements could include, but are not limited to, conducting additional or redesigned trials and procedures (e.g., additional patient muscle biopsies and dystrophin analyses), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, in the U.S., an FDA advisory committee or regulators may disagree with our data analysis, interpretations and conclusions at any point in the approval process, which could negatively impact the approval of our NDA or result in a decision by the Company not to proceed with an NDA submission for a product candidate based on feedback from regulators.
- We may not have the resources required to meet regulatory requirements and successfully navigate what is generally a lengthy, expensive and extensive approval process for commercialization of drug product candidates. Any failure on our part to respond to these requirements in a timely and satisfactory manner could significantly delay or negatively impact confirmatory study timelines and/or the development plans we have for the exon 53- and exon 45-skipping or other product candidates. Responding to requests from regulators and meeting requirements for clinical studies, submissions and approvals may require substantial personnel, financial or other resources, which, as a small biopharmaceutical company, we may not be able to obtain in a timely manner or at all. In addition, our ability to respond to requests from regulatory authorities that involve our agents, third party vendors and associates may be complicated by our own limitations and those of the parties we work with. It may be difficult or

impossible for us to conform to regulatory guidance or successfully execute our product development plans in response to regulatory guidance, including guidance related to clinical trial design with respect to any NDA or MAA submissions.

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

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

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

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

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

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

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

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

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

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

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

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

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

and sequestration, among other reasons, the viability of the government and its agencies as a partner for further development of our Ebola and Marburg programs, or other programs, is uncertain. The options for us to further develop product candidates that were previously developed under contracts with the U.S. government with third parties may be limited or difficult in certain respects given that, after termination or expiration of a U.S. government contract, the government has broad license rights in intellectual property developed under such contract. Therefore, the U.S. government may have the right to develop all or some parts of product candidates that we have developed under a U.S. government contract after such contract has terminated or expired.

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

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

- recruit and retain employees, consultants or contractors with the required level of expertise;
- recruit and retain sufficient patients needed to conduct a clinical trial;

- enroll and retain participants, which is a function of many factors, including the size of the relevant population, the proximity of participants to clinical sites, activities of patient advocacy groups, the eligibility criteria for the trial, the existence of competing clinical trials, the availability of alternative or new treatments, side effects from the therapy, lack of efficacy, personal issues and ease of participation;
- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the contract research organizations ("CROs") involved in the clinical trial;
- negotiate contracts and other related documents with clinical trial parties and institutional review boards, such as informed consents, CRO agreements and site agreements, which can be subject to extensive negotiations that could cause significant delays in the clinical trial process, with terms possibly varying significantly among different trial sites and CROs and possibly subjecting the Company to various risks;
- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;
- manage or resolve unforeseen adverse side effects during a clinical trial;
- conduct the clinical trials in a cost-effective manner, including managing foreign currency risk in clinical trials conducted in foreign jurisdictions and cost increases due to unforeseen or unexpected complications such as enrollment delays, or needing to outsource certain Company functions during the clinical trial; and
- execute clinical trial designs and protocols approved by regulatory authorities without deficiencies.

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

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

We incurred an operating loss of $45.7 million for the three months ended September 30, 2017. Our accumulated deficit was $1.2 billion as of September 30, 2017. Although we launched EXONDYS 51 in the U.S. in September 2016, we believe that it will take us some time to attain profitability and positive cash flow from operations. We have not yet generated significant revenues from product sales and have generally incurred expenses related to research and development of our technologies and product candidates, from general and administrative expenses that we have incurred while building our business infrastructure. We anticipate that our expenses will increase substantially if and/or as we:

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

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

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

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

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

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

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

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

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

- requiring us to maintain pledge cash and certain other assets in favor of MidCap during the term of the agreements;
- limiting our flexibility in planning for, or reacting to, changes in our business and our industry;
- placing us at a competitive disadvantage compared to our competitors who have less debt or competitors with comparable debt at more favorable interest rates;

- limiting our ability to borrow additional amounts for working capital, capital expenditures, research and development efforts, acquisitions, debt service requirements, execution of our business strategy and other purposes; and
- resulting in an acceleration of the maturity of such term loans upon the occurrence of a material adverse change or another default under the agreements with MidCap.

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

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

Our consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the U.S. The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. Such estimates and judgments include revenue recognition, inventory, valuation of stock-based awards, research and development expenses and income tax. We base our estimates on historical experience, facts and circumstances known to us and on various other assumptions that we believe to be reasonable under the circumstances. We cannot provide assurances, however, that our estimates, or the assumptions underlying them, will not change over time or otherwise prove inaccurate. If this is the case, we may be required to restate our consolidated financial statements, which could, in turn, subject us to securities class action litigation. Defending against such potential litigation relating to a restatement of our consolidated financial statements would be expensive and would require significant attention and resources of our management. Moreover, our insurance to cover our obligations with respect to the ultimate resolution of any such litigation may be inadequate. As a result of these factors, any such potential litigation could have a material adverse effect on our financial results and cause our stock price to decline, which could in turn subject us to securities class action litigation.

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.

On April 24, 2017, Dr. Edward M. Kaye informed our board of directors of his intention to resign as President and Chief Executive Officer. On June 26, 2017, Dr. Kaye tendered his resignation as President and Chief Executive Officer effective on that date. Also on June 26, 2017, due to Dr. Kaye's resignation as President and Chief Executive Officer and as required by the terms of his employment agreement, Dr. Kaye tendered his resignation as a director of the Company, effective upon a date to be determined by the board of directors or the board's Nominating and Corporate Governance Committee. On June 26, 2017, the board of directors also appointed Douglas S. Ingram to serve as the Company's President and Chief Executive Officer. Mr. Ingram was also elected to the

board of directors as a Group I director who will hold office as a director until the Company's 2018 annual meeting of stockholders or until his successor is earlier elected. On August 17, 2017, our board of directors accepted Dr. Kaye's resignation as a director and approved his engagement as an independent consultant to our company.

While Dr. Kaye serves us in an advisory capacity to ensure a smooth transition, we cannot guarantee that the transition to the new Chief Executive Officer will be smooth, successful or will not result in a negative impact to the Company. Leadership transitions can be inherently difficult to manage and may cause uncertainty or a disruption to our business or may increase the likelihood of turnover in other key officers and employees. If we lose the services of one or more of our senior management or key employees, or if one or more of them decides to join a competitor or otherwise to compete with us, our business could be harmed.

Our business operations are dependent upon our Chief Executive Officer to learn his new role.

We have a new Chief Executive Officer who started on June 26, 2017. As Mr. Ingram gains experience in his role, we could experience inefficiencies or a lack of business continuity due to loss of historical knowledge and a lack of familiarity with business processes, operating requirements, policies and procedures, and key information technologies and related infrastructure used in our day-to-day operations and we may experience additional costs as the new Chief Executive Officer learns his role and gains necessary experience. It is important to our success that the Chief Executive Officer quickly adapts to and excels in his new role. If he is unable to do so, our business, financial results and stock price could be materially 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 depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our technologies, product and product candidates, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing on the

proprietary rights of third parties.

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

We may not be able to obtain and maintain patent protection for our product or product candidates necessary to prevent competitors from commercializing competing product candidates. Our patent rights might be challenged, invalidated, circumvented or otherwise not provide any competitive advantage, and we might not be successful in challenging the patent rights of our competitors through litigation or administrative proceedings. Additionally, in order to maintain or obtain freedom to operate for our products and product candidates, we may incur significant expenses, including those associated with entering into agreements with third parties that require milestone and royalty payments. For example, in July 2017, we and The University of Western Australia on the one hand, and

the BioMarin Parties and AZL on the other hand, executed a Settlement Agreement pursuant to which all existing efforts pursuing ongoing litigation, opposition and other administrative proceedings would be stopped as between the Settlement Parties and the Settlement Parties would cooperate to withdraw the Actions before the European Patent Office (except for actions involving third parties), the United States Patent and Trademark Office ("USPTO"), the U.S. Court of Appeals for the Federal Circuit and the High Court of Justice of England and Wales, except for the cross-appeal of the Interlocutory Decision of the Opposition Division dated April 15, 2013 of the European Patent Office of EP 1619249B1 in which we withdrew our appeal and the BioMarin Parties and AZL will continue with its appeal, with us having the right to provide input on the appeal. Any adverse rulings on the appeal could come at any time and, if negative, could adversely affect our business and result in a decline in our stock price. Defending our patent positions may continue to require significant financial resources and could negatively impact other Company objectives. In addition, the expected benefits and opportunities related to the Settlement Agreement and the License Agreement may not be realized or may take longer to realize than expected due to challenges and uncertainties regarding the sales of EXONDYS 51, the research and development of future exon-skipping products, BioMarin's retained rights to convert the exclusive patent license under the Settlement Agreement to a co-exclusive license, BioMarin continuing certain oppositions and appeals, and patent oppositions that have been filed by other third parties, and patent oppositions and other patent challenges that may be filed by third parties in the future.

The DMD patent landscape is continually evolving, and we may be able to assert that certain activities engaged in by third parties infringe on our current or future patent rights. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights. As such, the patents and patent applications that we own or license and rely on for exclusivity for our product candidates may be challenged. In the U.S., our patents may be challenged in an Inter Partes Review proceeding or other related proceeding. In other countries, other procedures are available for a third party to challenge the validity of our patent rights. For instance, we have rights to European Patent No. 2206781, which protects SRP-4053. This patent was opposed at the European Patent Office. We filed our response to the opponent's opposition statement and the European Patent Office issued a summons for oral proceeding. The outcome and timing of a final written decision from the European Patent Office 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. 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 follow on exon-skipping product candidates. For example, we believe that companies including Alnylam Pharmaceuticals, Inc., Ionis Pharmaceuticals, Inc. (formerly Isis Pharmaceuticals, Inc.), Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Wave Life Sciences, Daiichi Sankyo and Nippon Shinyaku Co. Ltd. share a focus on RNA-targeted drug discovery and development. Competitors with respect to EXONDYS 51 or our product candidates include Nippon Shinyaku, Daiichi Sankyo, Wave Life Sciences and Shire plc; and other companies such as PTC have also been working on DMD programs. Additionally, several companies and institutions have entered into collaborations or other agreements for the development of product candidates, including mRNA, gene (CRIPSR and AAV, among others) and small molecule therapies that are potential competitors for therapies being developed in the muscular dystrophy, neuromuscular and rare disease space, including, but not limited to, Pfizer, Inc., Bristol-Myers Squibb, Roche, Biogen Idec, Inc., Ionis Pharmaceuticals, Inc., Alexion Pharmaceuticals, Inc., Sanofi, Eli Lilly, Alnylam, Moderna Therapeutics, Inc., Summit, Akashi, Catabasis, and Oxford University. Although BioMarin announced on May 31, 2016 its intent to discontinue clinical and regulatory development of drisapersen as well as its other clinical stage candidates, BMN 044, BMN 045 and BMN 053, then-currently in Phase 2 studies for distinct forms of DMD, it further announced its intent to continue to explore the development of next generation oligonucleotides for the treatment of DMD.

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

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

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

- obtain regulatory approval more quickly;
- have access to more manufacturing capacity;
- develop products that are more convenient and easier to administer;
- form more advantageous strategic alliances; or
- establish superior intellectual property positions.

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

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

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

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

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

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

damage our reputation, which could adversely affect our business.

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

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

Risks Related to Our Common Stock

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

The market prices for and trading volumes of securities of biotechnology companies, including our securities, has historically been volatile. Our stock has had significant swings in trading prices, in particular in connection with our public communications regarding feedback received from regulatory authorities. For example, over the last eighteen months, our stock has increased as much as 74% in a single day or decreased as much as 44% in a single day. The market has from time to time experienced significant price and volume fluctuations unrelated to the operating performance of particular companies. The market price of our common stock may fluctuate significantly due to a variety of factors, including but not limited to:

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

- comments by securities analysts;

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

- changes in senior management; or

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

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

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

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

- when the board is comprised of six or more directors, classification of our board of directors into two classes, with one class elected each year;

- directors may only be removed for cause by the affirmative vote of a majority of the voting power of all the then-outstanding shares of voting stock;

- prohibition of cumulative voting of shares in the election of directors;

- right of the board of directors to elect directors to fill a vacancy created by the expansion of the board of directors or the resignation, death, disqualification or removal of a director;

- express authorization of the board of directors to make, alter or repeal our bylaws;

- prohibition on stockholder action by written consent;

- advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at stockholder meetings;

- the ability of our board of directors to authorize the issuance of undesignated preferred stock, the terms and rights of which may be established and shares of which may be issued without stockholder approval, including rights superior to the rights of the holders of common stock; and

- a super-majority (66 2/3%) of the voting power of all of the then-outstanding shares of capital stock are required to amend, rescind, alter or repeal our bylaws and certain provisions of our certificate of incorporation.

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

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

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

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

As of September 30, 2017, there were approximately 64.6 million shares of common stock outstanding and outstanding awards to purchase 9.8 million shares of common stock under various incentive stock plans. Additionally, as of September 30, 2017, there were approximately 2.3 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan, approximately 0.2 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan and approximately 0.7 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 1, 2017 By: /s/ DOUGLAS S. INGRAM
Douglas S. Ingram
President and Chief Executive Officer
(Principal Executive Officer)

Date: November 1, 2017 By: /s/ SANDESH MAHATME
Sandesh Mahatme
Executive Vice President,
Chief Financial Officer and
Chief Business Officer
(Principal Financial and Accounting Officer)

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated File Form	File No.	Exhibit	Filing Date	Provided Herewith
10.1†	Consulting Agreement dated August 17, 2017 by and between Sarepta Therapeutics, Inc. and Dr. Edward M. Kaye					X
10.2†	Offer Letter dated August 28, 2017 by and between Sarepta Therapeutics, Inc. and Guriqbal S. Basi					X
10.3†	Letter Agreement by and between Sarepta Therapeutics, Inc. and Guriqbal S. Basi dated September 25, 2017					X
10.4†	Letter Agreement by and between Sarepta Therapeutics, Inc. and Catherine Stehman-Breen dated September 26, 2017					X
31.1	Certification of the Company's Chief Executive Officer, Douglas S. Ingram, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company's Executive Vice President, Chief Financial Officer and Chief Business Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1*	Certification of the Company's Chief Executive Officer, Douglas S. Ingram, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2*	Certification of the Company's Executive Vice President, Chief Financial Officer and Chief Business Officer, Sandesh Mahatme, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	XBRL Instance Document.					X
101.SCH	XBRL Taxonomy Extension Schema Document.					X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.					X

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

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.