Sarepta Therapeutics, Inc.
Form 10-Q
October 31, 2018

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, 2018

OR

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

Commission file number 001-14895

SAREPTA THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	93-0797222
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
215 First Street, Suite 415	
Cambridge, MA	02142
(Address of principal executive offices)	(Zip Code)

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

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

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

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

Large accelerated filer	Accelerated filer
Non-accelerated filer	Smaller Reporting Company
Emerging growth company	

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

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

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

Common Stock with $0.0001 par value	66,823,624
(Class)	(Outstanding as of October 26, 2018)

SAREPTA THERAPEUTICS, INC.

FORM 10-Q

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PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

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

	As of September 30, 2018	As of December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$209,702	$599,691
Short-term investments	583,158	479,369
Accounts receivable	48,601	29,468
Inventory	115,816	83,605
Other current assets	54,800	36,511
Total current assets	1,012,077	1,228,644
Property and equipment, net of accumulated depreciation of $25,224 and $18,022 as of September 30, 2018, and December 31, 2017, respectively	76,841	43,156
Intangible assets, net of accumulated amortization of $5,532 and $4,145 as of September 30, 2018, and December 31, 2017, respectively	15,324	14,355
Other assets	78,664	21,809
Total assets	$1,182,906	$1,307,964
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$20,408	$8,467
Accrued expenses	88,687	68,982
Current portion of long-term debt	—	6,175
Deferred revenue	3,303	3,316
Other current liabilities	1,995	1,392
Total current liabilities	114,393	88,332
Long-term debt	415,446	424,876
Deferred rent and other	13,219	5,539
Total liabilities	543,058	518,747
Commitments and contingencies (Note 16)		

Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 99,000,000 shares authorized; 66,693,348 and 64,791,670 issued and outstanding at September 30, 2018, and December 31, 2017, respectively	7	6
Additional paid-in capital	2,077,864	2,006,598
Accumulated other comprehensive income (loss)	8	(379)
Accumulated deficit	(1,438,031)	(1,217,008)
Total stockholders' equity	639,848	789,217
Total liabilities and stockholders' equity	$1,182,906	$1,307,964

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(unaudited, in thousands, except per share amounts)

	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2018	2017	2018	2017
Revenues:				
Product, net	$ 78,486	$ 45,954	$ 216,619	$ 97,307
Total revenues	78,486	45,954	216,619	97,307
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	$ 8,741	3,078	$ 21,058	3,807
Research and development	86,584	34,239	255,636	122,266
Selling, general and administrative	53,044	28,176	143,541	90,461
EXONDYS 51 litigation and license charges	—	25,588	—	28,427
Amortization of in-licensed rights	216	780	649	837
Total costs and expenses	148,585	91,861	420,884	245,798
Operating loss	(70,099)	(45,907)	(204,265)	(148,491)
Other (loss) income:				
Gain from sale of Priority Review Voucher	—	—	—	125,000
Interest (expense) income and other, net	(6,968)	184	(16,671)	703
Other (loss) income	(6,968)	184	(16,671)	125,703
Loss before income tax (benefit) expense	(77,067)	(45,723)	(220,936)	(22,788)
Income tax (benefit) expense	(674)	2,011	87	3,902
Net loss	(76,393)	(47,734)	(221,023)	(26,690)
Other comprehensive income:				
Unrealized gain on cash equivalents and short-term investments	369	26	387	108
Total other comprehensive income	369	26	387	108
Comprehensive loss	$ (76,024)	$ (47,708)	$ (220,636)	$ (26,582)
Net loss per share - basic and diluted	$ (1.15)	$ (0.78)	$ (3.38)	$ (0.47)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	66,209	61,528	65,454	57,166

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,	
	2018	2017
Cash flows from operating activities:		
Net loss	$ (221,023)	$ (26,690)
Adjustments to reconcile net loss to cash flows from operating activities:		
Gain from sale of Priority Review Voucher	—	(125,000)
Depreciation and amortization	8,718	5,968
Amortization of investment discount	(4,742)	(344)
Loss from debt extinguishment	2,322	—
Non-cash interest expense	15,206	200
Loss on disposal of assets	94	792
Stock-based compensation	37,289	23,099
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable	(19,133)	(19,523)
Net increase in inventory	(32,211)	(51,880)
Net increase in other assets	(84,344)	(7,319)
Net increase (decrease) in accounts payable, accrued expenses, deferred revenue and other liabilities	31,584	(241)
Net cash used in operating activities	(266,240)	(200,938)
Cash flows from investing activities:		
Purchase of property and equipment	(40,954)	(8,101)
Purchase of intangible assets	(2,633)	(8,591)
Purchase of available-for-sale securities	(651,387)	(100,348)
Maturity and sale of available-for-sale securities	562,575	296,225
Proceeds from sale of Priority Review Voucher	—	125,000
Purchases of restricted investment	(353)	—
Maturity of restricted investment	—	10,695
Net cash (used in) provided by investing activities	(132,752)	314,880
Cash flows from financing activities:		
Proceeds from July 2017 Term Loan, net of cash debt issuance costs	—	29,620
Repayment of June 2015 and July 2017 Term Loan	(30,000)	(15,000)
Proceeds from revolving line of credit	217,722	24,000
Repayment of revolving line of credit	(218,631)	(23,008)
Repayments on mortgage loans	(1,265)	(81)
Payment of debt extinguishment	(1,990)	—
Proceeds from sale of common stock, net of offering costs	—	353,959
Proceeds from exercise of stock options and purchase of stock under the Employee Stock	43,031	11,779

Purchase Program		
Net cash provided by financing activities	8,867	381,269
(Decrease) increase in cash and cash equivalents	(390,125)	495,211
Cash, cash equivalents and restricted cash:		
Beginning of period	599,827	122,556
End of period	$ 209,702	$ 617,767
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 209,702	$ 617,630
Restricted cash in other assets	—	137
Total cash, cash equivalents and restricted cash	$ 209,702	$ 617,767
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 6,861	$ 924
Supplemental schedule of non-cash investing activities and financing activities:		
Shares withheld for taxes	$ 9,053	$ 1,791
Reclassification of long term investments to short term investments	$ 9,980	$ —
Reclassification of revolving line of credit balance to other receivable	$ 683	$ —
Intangible assets included in accrued expenses	$ 234	$ 258
Asset held for sale	$ —	$ 1,529
Debt issuance costs related to the term loans included in accrued expenses	$ —	$ 600
Offering costs related to equity offerings included in accrued expenses	$ —	$ 25
Property and equipment included in accrued expenses	$ 2,515	$ 385

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

1. ORGANIZATION AND NATURE OF BUSINESS

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

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

In November 2016, the Company submitted a marketing authorization application ("MAA") for eteplirsen to the European Medicines Agency ("EMA") and the application was validated in December 2016. On June 1, 2018, the Committee for Medicinal Products for Human Use ("CHMP") of the EMA adopted a negative opinion for eteplirsen. On September 21, 2018, the Company announced that the CHMP has confirmed its negative opinion for eteplirsen. The Company expects the European Commission to adopt the CHMP opinion by year-end 2018.

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

As of September 30, 2018, the Company had approximately $793.9 million of cash, cash equivalents and investments, consisting of $209.7 million of cash and cash equivalents, $583.2 million of short-term investments, and $1.0 million of long-term restricted investment. The Company believes that its balance of cash, cash equivalents and investments as of the date of the issuance of this report is sufficient to fund its current operational plan for at least the next twelve months, though it may pursue additional cash resources through public or private debt and equity financings, seek additional government contracts and establish collaborations with or license its technology to other companies.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES AND RECENT ACCOUNTING PRONOUNCEMENTS

Basis of Presentation

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

Estimates and Uncertainties

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

Concentration of Credit Risk

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

As of September 30, 2018, the majority of the Company's accounts receivable arose from product sales in the U.S. and all customers have standard payment terms which generally require payment within 30 to 60 days. Outside of the U.S., the payment terms range between 30 and 120 days. Three individual customers accounted for 43%, 36% and 18% of net product revenues for the nine months ended September 30, 2018 and 55%, 24% and 12% of accounts receivable from product sales as of September 30, 2018. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profile. As of September 30, 2018, the Company believes that such customers are of high credit quality.

As of September 30, 2018 the Company's cash equivalents and investments were concentrated at two financial institutions in the U.S., 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, 2017.

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

Product Revenues

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

Variable Consideration

Product revenues are recorded at the net sales price ("transaction price") which includes estimated reserves for variable consideration, such as Medicaid rebates, governmental chargebacks, including Public Health Service ("PHS") chargebacks, prompt payment discounts, co-pay assistance and distribution fees. These reserves are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable (if no payment is required by the Company) or a current liability (if a payment is required by the Company). These reserves reflect the Company's best estimates of the amount of consideration to which it is entitled based on the terms of the contracts. Additional details relating to variable consideration follows:

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

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

Recent Accounting Pronouncements

In January 2016, the Financial Accounting Standards Board ("FASB") issued ASU No. 2016-01, "Financial Instruments - Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities". Under the new guidance, entities are required to measure equity investments (except those accounted for under the equity method, those that result in consolidation of the investee and certain other investments) at fair value and recognize any changes in fair value in net income. However, for equity investments that do not have readily determinable fair values and do not qualify for the existing practical expedient available in ASC Topic 820, "Fair Value Measurement" to estimate fair value using the net asset value per share (or its equivalent) of the investment, the guidance provides a new measurement alternative. Entities may choose to measure those investments at cost, less any impairment, plus or minus changes resulting from observable price changes in orderly transactions for the identical or a similar investment of the same issuer. The Company adopted this ASU on January 1, 2018, which did not have a material impact on its consolidated financial statements.

In February 2016, the FASB issued ASU No. 2016-02, “Leases (Topic 842)”, which supersedes ASC 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. In July 2018, the FASB issued ASU No. 2018-10, “Codification Improvement to Topic 842, Leases” and ASU No. 2018-11, “Leases (Topic 842), Targeted Improvements”. ASU No. 2018-10 made 16 technical corrections to the new leases standard, clarifying certain inconsistencies in the guidance. ASU No. 2018-11 provides entities with a new transition method that allows them to use the effective date of the new leases standard as the date of initial application on transition. Companies that elect this transition method will (1) not adjust their comparative period financial information for the effects of ASC 842; (2) not make the new required lease disclosures for periods before the effective date;

and (3) carry forward their ASC 840 disclosure for comparative periods. Additionally, this update allows lessors to make an accounting policy election by class of underlying assets to not separate lease and non-lease components if specified criteria are met. ASU No. 2016-02, ASU No. 2018-10 and ASU No. 2018-11 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The adoption of these standards is expected to have an impact on the amount of the Company's assets and liabilities presented. The Company expects to utilize the new transition method described in ASU No. 2018-11 and use the effective date as the Company's date of initial application for the new standard. The Company expects to elect the available package of practical expedients in transition which would allow it to not re-assess whether existing or expired arrangements contain a lease, the lease classification of existing or expired leases, or whether previous initial direct costs would qualify for capitalization under the new lease standard. As of September 30, 2018, the Company has not elected to early adopt the guidance or determined the effect that the adoption of this guidance will have on its consolidated financial statements.

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

In June 2018, the FASB issued ASU No. 2018-07, "Compensation - Stock Compensation (Topic 718), Improvements to Nonemployee Share-Based Payment Accounting." This ASU expands the scope of Topic 718 to include share based payment transactions for acquiring goods and services from nonemployees. An entity should apply the requirements of Topic 718 to nonemployee awards except for specific guidance on inputs to an option pricing model and the attribution of cost. ASU No. 2018-07 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted, although no earlier than the adoption date of Topic 606. The Company elected to early adopt this ASU in the quarter ended June 30, 2018, which did not have a material impact on its consolidated financial statements.

In August 2018, the FASB issued ASU No. 2018-13, "Fair Value Measurement (Topic 820), Disclosure Framework – Changes to the Disclosure Requirements for Fair Value Measurement". This ASU removed the following disclosure requirements: (1) the amount of and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy; (2) the policy for timing of transfers between levels; and (3) the valuation processes for Level 3 fair value measurements. Additionally, this update added the following disclosure requirements: (1) the changes in unrealized gains and losses for the period included in other comprehensive income for recurring Level 3 fair value measurements held at the end of the reporting period; (2) the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements. For certain unobservable inputs, an entity may disclose other quantitative information (such as the median or arithmetic average) in lieu of the weighted average if the entity determines that other quantitative information would be a more reasonable and rational method to reflect the distribution of unobservable inputs used to develop Level 3 fair value measurements. ASU No. 2018-13 will be effective for fiscal years beginning after December 15, 2019 with early adoption permitted. As of September 30, 2018, the Company has not elected to early adopt this guidance but does not expect that the adoption of this guidance will have a material effect on its consolidated financial statements.

In August 2018, the FASB issued ASU No. 2018-15, "Intangibles – Goodwill and Other – Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That is a Service Contract". This ASU requires a customer in a cloud computing arrangement (i.e., hosting arrangement) that is a service contract to follow the internal-use software guidance contained in ASC Subtopic 350-40 to determine which implementation costs to capitalize as assets or expense as incurred. Capitalized implementation costs related to a hosting arrangement that is a service contract will be amortized over the term of the hosting arrangement, beginning when the module or component of the hosting arrange is ready for its intended use. ASU No. 2018-15 will be effective for fiscal years beginning after December 15, 2019, with early adoption permitted. As of

September 30, 2018, the Company has not elected to early adopt this guidance but believes that the adoption of this guidance will not have a material effect on its consolidated financial statements.

3. COLLABORATION, LICENSE AND MANUFACTURING AGREEMENTS

Lacerta Therapeutics

On August 8, 2018 (the "Effective Date"), the Company entered into a License, Development and Option Agreement (the "License Agreement") with Lacerta Therapeutics, Inc. ("Lacerta"). Pursuant to the License Agreement, the Company licensed in exclusive worldwide rights to develop, manufacture and commercialize a pre-clinical Pompe product candidate (the "Pompe License"). Lacerta also granted the Company exclusive options to enter into exclusive License Agreements to develop, manufacture and commercialize other gene therapy product candidates for Sanfilipo syndrome and L-Amino Acid Decarboxylase Deficiency for additional consideration of $42.0 million (collectively, the "Options") when (and if) the Options are exercised. Additionally, the Company may be liable for up to approximately $44.0 million in development, regulatory and sales milestones associated with the Pompe License and may be required to make a high-single-digit royalty payments based on net sales of the Pompe product subsequent to its commercialization.

Concurrently with the execution of the License Agreement, the Company entered into a Series A Preferred Stock Purchase Agreement (the "Purchase Agreement") with Lacerta. Under the Purchase Agreement, the Company purchased approximately 4.5 million shares of Series A preferred stock issued by Lacerta.

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

The Company made an up-front payment of $38.0 million to Lacerta in consideration of both the License Agreement and the Purchase Agreement. This payment was allocated to the fair value of the Series A preferred stock investment, the Pompe License and the Options based on their respective relative fair values on the Effective Date. The fair value of the Options were determined using an option pricing model, whereas the Series A preferred stock investment was determined using a cost approach corroborated by the Black-Scholes option pricing model. The fair value of the Pompe License was determined using a discounted cash flow model under the income approach. Accordingly: (i) $30.0 million was allocated to the Series A preferred stock investment, (ii) $8.0 million was allocated to the Pompe License, and (iii) no amount was allocated to the Options as they are far out of money and were determined to have a fair value of zero. fThe Series A preferred stock investment was initially measured at cost and classified as an other non-current asset in the accompanying unaudited condensed consolidated balance sheets. Subsequently, changes in the carrying value of the investment will be reported as a component of earnings whenever there are observable price changes in orderly transactions for identical or similar investments of Lacerta in the future. The amount allocated to the Pompe License represents rights to potential future benefits associated with ongoing research and development activities that have no alternative future use. Accordingly, this amount has been recorded as research and development expense in the accompanying unaudited condensed consolidated statements of operations and comprehensive loss for the three and nine months ended September 30, 2018.

Myonexus Therapeutics

In May 2018, the Company entered into a Warrant to Purchase Common Stock Agreement ("Warrant Agreement") with Myonexus Therapeutics, Inc. ("Myonexus"). Pursuant to the terms of the Warrant Agreement, the Company made an up-front payment of $60.0 million to purchase an exclusive option to acquire Myonexus for $200.0 million plus sales-related and regulatory-related contingent payments. Prior to the exercise of the option to acquire Myonexus, the Company may be required to make additional development milestone payments to Myonexus of up to $45.0 million over an approximately two-year evaluation period. The Company considered whether it would have to consolidate the operations of Myonexus and concluded that, while Myonexus is a variable interest entity, the Company is not the primary beneficiary as it does not have the power to direct the activities that would most significantly impact the economic performance of Myonexus.

As of September 30, 2018, the Company made an up-front payment of $60.0 million and a milestone payment of $10.0 million to Myonexus corresponding to execution of the Warrant Agreement in May 2018 and achievement of a development milestone in September 2018, respectively. Prior to regulatory approval, considerations paid to Myonexus represent rights to potential future benefits associated with Myonexus's ongoing research and development activities, which have not reached technological feasibility and have no alternative future use. Accordingly, the Company recorded $10.0 million and $70.0 million for the three and nine months ended September 30, 2018, respectively, as research and development expense in the accompanying unaudited condensed consolidated statements of operations and comprehensive loss.

Brammer Bio MA, LLC

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

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

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

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

As of September 30, 2018, the Company has made payments totaling $35.0 million to Brammer under the Brammer Manufacturing Agreement consisting of: (i) the $20.0 million advance payment made in June 2018, (ii) the first $5.0 million process equipment fee paid in July 2018, and (iii) the first two reduced quarterly capacity access fee payments of $5.0 million made in July 2018 and September 2018. Of the cumulative amount paid of $35.0 million, $2.2 million was recorded as an other current asset and $32.8 million as an other non-current asset, in the accompanying unaudited condensed consolidated balance sheets.

The advance payment and process equipment fee will be amortized over their expected economic benefit to research and development expense, prior to regulatory approval of the related product, commencing upon the first batch delivery to the Company, which is currently estimated to occur in July 2019. Upon regulatory approval, amortization expense will be classified to cost of sales. Capacity access fee payments made prior to the first batch delivery to the Company will be capitalized and amortized to expense in a manner similar to the advance payment and process equipment fee. Capacity access fee payments made subsequent to the first batch delivery to the Company will be expensed as incurred to research and development expense, prior to regulatory approval of the related product. Upon regulatory approval, the expense associated with capacity access fee payments will be classified to cost of sales. In the event the Company does not expect services under the Brammer Manufacturing Agreement to be rendered, the capitalized payments will be charged to expense.

4. FAIR VALUE MEASUREMENTS

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

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

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

	Fair Value Measurement as of September 30, 2018			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$85,321	$85,321	$—	$—
Commercial paper	89,795	—	89,795	—
Government and government agency bonds	510,028	510,028	—	—
Corporate bonds	83,261	83,261	—	—
Strategic investment	30,000	—	—	30,000
Certificates of deposit	1,001	1,001	—	—
Total	$799,406	$679,611	$89,795	$30,000

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

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

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

The Company's assets with fair value categorized as Level 3 within the fair value hierarchy consists of a strategic investment in Series A preferred stock of Lacerta as more fully described in Note 3, Collaboration, License and Manufacturing Agreements. The fair value of the asset is based on a cost approach corroborated by the Black-Scholes option pricing model. The most significant assumptions in the option pricing model include historical volatility of similar public companies, estimated term through Lacerta's potential exit and a risk free rate based on certain U.S. Treasury rates as of the Effective Date of the License Agreement with Lacerta.

The carrying amounts reported in the unaudited condensed consolidated balance sheets for cash and cash equivalents, accounts receivable, accounts payable, and revolving line of credit approximated fair value because of the immediate

or short-term maturity of these financial instruments. The carrying amounts for the term loan approximated fair value based on market activity for other debt instruments with similar characteristics and comparable risk.

5. CASH, CASH EQUIVALENTS AND MARKETABLE SECURITIES

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

	As of September 30, 2018	As of December 31, 2017
	(in thousands)	
Money market funds	$85,321	$352,370
Corporate bonds	—	16,720
Government and government agency bonds	99,926	49,972
Total	$185,247	$419,062

It is the Company's policy to mitigate credit risk in its financial assets by maintaining a well-diversified portfolio that limits the amount of exposure as to maturity and investment type. The weighted average maturity of the Company's available-for-sale securities as of September 30, 2018 and December 31, 2017 was approximately two and seven months, respectively.

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

	As of September 30, 2018			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$109,776	$ —	$ —	109,776
Commercial paper	89,795	—	—	89,795
Government and government agency bonds	509,944	213	(129)	510,028
Corporate bonds	83,337	—	(76)	83,261
Total	$792,852	$ 213	$ (205)	792,860
As reported:				
Cash and cash equivalents	$209,576	$ 126	$ —	209,702
Short-term investments	583,276	87	(205)	583,158
Total	$792,852	$ 213	$ (205)	792,860

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

6. ACCOUNTS RECEIVABLE AND RESERVES FOR PRODUCT SALES

The Company’s accounts receivable arise from product sales, government research contracts and other grants. They are generally stated at the invoiced amount and do not bear interest. Revenues from product sales are recorded at the net sales price (transaction price), which includes estimates of variable consideration for which reserves are established and which result from Medicaid rebates, governmental chargebacks including Public Health Services chargebacks, prompt pay discounts, co-pay assistance and distribution fees. These reserves are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable (if no payments are required of us), including Public Health Services chargebacks, prompt pay discounts and certain distribution fees, or a current liability (if a payment is required of us), including Medicaid rebates, co-pay assistance and certain distribution fees.

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

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

	As of September 30, 2018	As of December 31, 2017
	(in thousands)	
Product sales, net of discounts and allowances	$47,809	$ 28,539
Government contract receivables	792	929
Total accounts receivable	$48,601	$ 29,468

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

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

	Chargebacks	Rebates	Prompt Pay	Other	Total
	(in thousands)				
Balance, as of December 31, 2017	$995	$6,959	$169	$464	$8,587
Provision	9,308	19,298	1,804	4,195	34,605
Payments/credits	(9,502)	(6,479)	(1,526)	(2,532)	(20,039)
Balance, as of September 30, 2018	$801	$19,778	$447	$2,127	$23,153

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, 2018	As of December 31, 2017
	(in thousands)	
Reduction to accounts receivable	$1,469	$ 1,285
Component of accrued expenses	21,684	7,302

Total reserves	$23,153	$ 8,587

7. INVENTORY

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

	As of September 30, 2018	As of December 31, 2017
	(in thousands)	
Raw materials	$68,689	$ 53,875
Work in progress	42,537	27,442
Finished goods	4,590	2,288
Total inventory	$115,816	$ 83,605

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

8. OTHER CURRENT ASSETS AND OTHER NON-CURRENT ASSETS

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

	As of September 30, 2018	As of December 31, 2017
	(in thousands)	
Manufacturing-related deposits and prepaids	$18,465	$ 18,650
Leasehold improvement receivable	10,787	—
Prepaid clinical and pre-clinical expenses	10,658	5,175
Prepaid maintenance and license fees	2,415	1,711
Prepaid research expenses	1,939	2,896
Prepaid commercial expenses	2,125	1,589
Asset held for sale	—	1,501
Other prepaids	3,537	2,726
Other	4,874	2,263
Total other current assets	$54,800	$ 36,511

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

	As of September 30, 2018	As of December 31, 2017
	(in thousands)	
Manufacturing-related deposits	$40,133	$ —
Strategic investments	30,000	—
Prepaid clinical expenses	4,594	7,488
Alternative minimum tax credit	2,881	3,315
Restricted investment	1,001	784
Long-term available-for-sale securities	—	9,980
Other	55	242
Total other non-current assets	$78,664	$ 21,809

9. ACCRUED EXPENSES

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

	As of September 30, 2018	As of December 31, 2017
	(in thousands)	
Accrued employee compensation costs	$21,915	$ 14,402
Product revenue related reserves	21,684	7,302
Accrued clinical and pre-clinical costs	13,778	15,975
Accrued professional fees	9,112	6,794
Accrued contract manufacturing costs	8,348	14,019
Accrued BioMarin royalties	4,002	2,846
Accrued interest expense	3,183	1,291
Accrued property and equipment	2,515	2,525
Accrued research costs	2,506	401
Accrued collaboration cost sharing	1,200	—
Accrued income taxes	—	943
Other	444	2,484
Total accrued expenses	$88,687	$ 68,982

10. INDEBTEDNESS

2024 Convertible Notes

In November 2017, the Company issued $570.0 million senior notes due on November 15, 2024 (the "2024 Notes"). The 2024 Notes were issued at face value and bear interest at the rate of 1.50% per annum, payable semi-annually in cash on each May 15 and November 15, commencing on May 15, 2018. There are no principal payments due prior to maturity. Upon conversion, the Company may pay cash, shares of its common stock or a combination of cash and stock, as determined by the Company in its discretion. The 2024 Notes may be convertible into 7,763,552 shares of the Company's common stock under certain circumstances prior to maturity at a conversion rate of 13.621 shares per $1,000 principal amount of the 2024 Notes, which represents a conversion price of $73.42 per share. The Company recorded a total debt discount of $171.8 million upon issuance of the 2024 Notes, consisting of an equity component of $161.2 million and debt issuance costs of $10.6 million. The debt discount is being amortized under the effective interest method and recorded as additional non-cash interest expense over the life of the 2024 Notes. The effective interest rate on the liability component of the 2024 Notes for the year ended December 31, 2017 was 6.9%. The fair value of the 2024 Notes is $1.3 billion as of September 30, 2018. It is based on open market trades and is classified as level 1 in the fair value hierarchy.

Term Loan

In July 2017, the Company entered into an amended and restated credit agreement (the "Amended and Restated Credit and Security Agreement") which provides a term loan ("July 2017 Term Loan") of $60.0 million with MidCap Financial Trust ("MidCap"). Borrowings under the Amended and Restated Credit and Security Agreement bear interest at a rate per annum equal to 6.25%, plus the one-month London Interbank Offered Rate ("LIBOR"). Commencing on July 1, 2018, and continuing for the remaining thirty six months of the facility, the Company was 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.

Revolving Line of Credit

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

In September 2018, the Company terminated the Amended and Restated Credit and Security Agreement and the Revolving Credit Agreement with MidCap and paid off the remaining outstanding balance of principal and accrued and unpaid interest on the July 2017 Term Loan. As a result, the Company recorded a debt extinguishment loss of $2.3 million primarily related to the write-off of unamortized debt issuance costs and prepayment fees.

As of September 30, 2018, the Company recorded approximately $415.4 million as long-term debt on the unaudited condensed consolidated balance sheets related to the 2024 Notes. The following table summarizes the Company's debt facilities for the periods indicated:

	As of	As of

	September 30, 2018	December 31, 2017
	(in thousand)	
Par value of the 2024 Notes	570,000	570,000
Unamortized discount - equity component	(144,999)	(158,890)
Unamortized discount - debt issuance costs	(9,555)	(10,449)
Net carrying value of convertible debt	415,446	400,661
Other debt facilities	—	30,390
Net carrying value of total debt facilities	415,446	431,051

For the three months and nine months ended September 30, 2018, the Company recorded $10.7 million and $26.5 million in interest expense, respectively. For the three months and nine months ended September 30, 2017, the Company recorded $0.8 million and $1.2 million in interest expense, respectively.

11. RESTRUCTURING

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

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

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

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

12. STOCK-BASED COMPENSATION

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

	For the Three Months Ended September 30,				For the Nine Months Ended September 30,			
	2018 Grants	Weighted	2017 Grants	Weighted	2018 Grants	Weighted	2017 Grants	Weighted

		Average Grant Date Fair Value		Average Grant Date Fair Value		Average Grant Date Fair Value		Average Grant Date Fair Value
Stock options	359,182	$ 68.31	221,398	$ 21.07	2,011,863	$ 43.17	4,678,357	$ 14.49
Restricted stock units	5,990	$ 138.04	—	$ —	175,410	$ 76.27	181,029	$ 33.03
Restricted stock awards	10,500	$ 142.71	—	$ —	27,590	$ 98.57	341,500	$ 34.58

Stock-based Compensation Expense

For the three months ended September 30, 2018 and 2017, total stock-based compensation expense was $11.5 million and $6.9 million, respectively. For the nine months ended September 30, 2018 and 2017, total stock-based compensation expense was $37.3 million and $23.1 million, respectively. The increase in stock-based compensation expense for nine months was partially driven by increases in headcount and stock price, the achievement of a milestone related to the September 2016 restricted stock awards with performance conditions, as well as the impact of a revised forfeiture rate assumption for officers and members of our Board of Directors. 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,	
	2018	2017	2018	2017
	(in thousands)			
Research and development	$3,260	$1,812	$10,349	$5,881
Selling, general and administrative	8,224	5,110	26,940	17,218
Total stock-based compensation expense	$11,484	$6,922	$37,289	$23,099

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,	
	2018	2017	2018	2017
	(in thousands)			
Stock options	$9,007	$5,420	$27,362	$17,221
Restricted stock awards/units	1,987	954	8,654	4,408
Employee stock purchase plan	490	548	1,273	1,470
Total stock-based compensation expense	$11,484	$6,922	$37,289	$23,099

13. OTHER INCOME AND LOSS

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

	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2018	2017	2018	2017
	(in thousand)			
Interest expense	$(10,681)	$(802)	$(26,460)	$(1,325)
Interest income	1,477	438	5,236	813
Amortization of investment discount	2,295	433	5,123	693
Other (expense) income	(59)	115	(570)	522
Gain from sale of Priority Review Voucher	—	—	—	125,000
Total other (loss) income	$(6,968)	$184	$(16,671)	$125,703

14. INCOME TAXES

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

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

15. NET LOSS PER SHARE

Basic net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding. Diluted net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding. For the three and nine months ended September 30, 2018 and 2017, there were no differences between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive due to the net loss position and, therefore, would be excluded from the diluted net loss per share calculation.

	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2018	2017	2018	2017
	(in thousands, except per share amounts)			
Net loss	$(76,393)	$(47,734)	$(221,023)	$(26,690)
Weighted-average number of shares of common stock and common stock equivalents outstanding:				
Weighted-average number of shares of common stock outstanding for computing basic loss earnings per share	66,209	61,528	65,454	57,166
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	66,209	61,528	65,454	57,166
Net loss per share - basic and diluted	$(1.15)	$(0.78)	$(3.38)	$(0.47)

* For the three and nine months ended September 30, 2018 and 2017, stock options, RSAs, RSUs, stock appreciation rights (“SAR”) to purchase 9.2 million and 9.8 million shares of the Company’s common stock, respectively, were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

16. COMMITMENTS AND CONTINGENCIES

Lease Obligations

In April and September 2018, the Company entered into the seventh and eighth amendment, respectively, to its Cambridge, Massachusetts headquarters lease which extended the original term of the lease to September 30, 2025 and increased the total rental space to approximately 154,967 square feet.

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

	As of September 30, 2018 (in thousands)
2018 (September - December)	$ 1,827
2019	7,479
2020	7,510
2021	8,666
2022	8,956
Thereafter	25,604
Total minimum lease payments	$ 60,042

Manufacturing Obligations

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

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

	As of September 30, 2018 (in thousands)
2018 (September - December)	$ 45,463
2019	78,720
2020	45,940
2021	32,000
2022	40,000
Thereafter	160,000
Total manufacturing commitments	$ 402,123

Milestone Obligations

The Company has collaboration and license agreements in place for which it could be obligated to pay, in addition to the payment of up-front fees upon execution of the agreements, certain milestone payments as a product candidate proceeds from the submission of an investigational new drug application through approval for commercial sale and beyond. As of September 30, 2018, the Company may be obligated to make up to $289.8 million of future development, regulatory, and commercial milestone and up-front royalty payments associated with its collaboration and license agreements. For the three and nine months ended September 30, 2018, the Company recognized up-front and milestone payments of $18.0 million and 78.0 million, respectively, as research and development expense in the accompanying unaudited condensed consolidated statement of operations and comprehensive loss. For the three and nine months ended September 30, 2017, the Company recognized up-front and milestone payments of $0 and $22.0 million, respectively, as research and development expense in the accompanying unaudited condensed consolidated statement of operations and comprehensive loss.

Litigation

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

filed a notice of appeal on September 29, 2015, seeking review of the Court's March 31, 2015 order dismissing the case and the Court's September 2, 2015 order denying leave to amend. On January 27, 2016, Plaintiffs filed in the district court a motion for relief from judgment pursuant to Federal Rule of Civil Procedure 60(b)(2), arguing that the FDA Briefing Document published on or about January 15, 2016, was material and would have changed the Court's ruling. On February 26, 2016, the First Circuit stayed the appeal pending the district court's ruling on the 60(b)(2) motion. Defendants opposed the 60(b)(2) motion, and on April 21, 2016, the Court denied Plaintiffs' motion for relief from judgment. On May 19, 2016, Plaintiffs filed a motion to alter or amend the April 21, 2016 order pursuant to Federal Rule of Civil Procedure 59(e). On May 20, 2016, the Court denied Plaintiffs' motion, and Plaintiffs filed a notice of appeal of the Court's April 21, 2016 denial of their 60(b)(2) motion and May 20, 2016 denial of their 59(e) motion. On June 13, 2016, the First Circuit granted Plaintiffs' motion to consolidate the two appeals. Oral argument took place on March 7, 2017 and the First Circuit affirmed the District Court's dismissal of this case on August 22, 2017. Plaintiffs filed a Petition for Panel Rehearing and Rehearing En Banc, which the First Circuit denied on October 11, 2017. The period for filing a petition with the U.S. Supreme Court for a writ of certiorari has elapsed without a filing from the plaintiffs. As such, there is no risk of loss in connection with this litigation.

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

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

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

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

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17. SUBSEQUENT EVENTS

On October 8, 2018, the Company exercised an option to license Nationwide Children's Hospital's micro-dystrophin gene therapy program for the treatment of DMD under an agreement that was originally executed in December 2016. Upon exercise of the option, the Company made an up-front license payment of $1.0 million and may be liable for up to approximately $29.0 million in development, regulatory and sales milestones for each micro-dystrophin gene therapy product. Furthermore, the Company may be required to make a low-single-digit royalty payments based on net sales of micro-dystrophin gene therapy products subsequent to their commercialization.

On October 8, 2018, the Company entered into a manufacturing collaboration agreement ("Paragon Agreement") with Paragon Bioservices, Inc. ("Paragon"). Pursuant to the terms of the Paragon Agreement, Paragon agreed to provide the Company with dedicated access to clinical and commercial manufacturing capacity for its gene therapy programs. In return, the Company is required to make to Paragon: (i) a one-time $36.0 million payment payable in three installments through March 31, 2019, and (ii) additional payments totaling $2.0 million annually. The term of the Paragon Agreement will continue until the earlier of: (i) the latest expiration date of any commercial supply agreement in effect, or (ii) December 31, 2025. The Company also has the ability to terminate the Paragon Agreement prior to expiration, subject to potential additional financial considerations.

On October 15, 2018, the Company entered into a license and collaboration agreement for developing and commercializing LYS-SAF302, a gene therapy to treat Mucopolysaccharidosis type IIIA ("MPS IIIA") with Lysogene S.A. ("Lysogene"). Under the terms of the license and collaboration agreement, Lysogene will be responsible for completion of the pivotal trial, which is set to commence in the fourth quarter of 2018. The Company will have exclusive rights to commercialize LYS-SAF302 in the U.S. and all territories outside of Europe. Lysogene will retain exclusive commercial rights to LYS-SAF302 in Europe. The Company will be responsible for global manufacturing of LYS-SAF302 and will supply drug products to Lysogene for Europe. Lysogene also granted the Company certain option rights to an additional central nervous system targeted gene therapy candidate. Concurrently with the execution of the license and collaboration agreement, the Company entered into an equity investment agreement with Lysogene. Under the equity investment agreement, the Company purchased 950,606 shares of common stock issued by Lysogene. As a result of execution of the agreements, the Company was liable for a payment of $28.0 million to Lysogene related to the up-front license fee, the option fee, the equity investment and reimbursement to certain development activities. Additionally, the Company may be liable for up to approximately $102.8 million in development, regulatory and sales milestones associated with the license and collaboration agreement. Furthermore, the Company may be required to make tiered royalty payments based on net sales of the LYS-SAF302 product subsequent to its commercialization.

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

This section should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and the section contained in our Annual Report on Form 10-K for the year ended December 31, 2017 under the caption "Part II-Item 7 — Management's Discussion and Analysis of Financial Condition and Results of Operations". This discussion contains certain forward-looking statements, which are often identified by words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may," "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 expectations regarding the continued growth of our business operations due, in part, to the commercialization of EXONDYS 51® (eteplirsen) Injection ("EXONDYS 51");
- our technologies and programs, including those with strategic partners, and their respective potential benefits, including the potential of our phosphorodiamidate morpholino oligomer ("PMO") based compounds to reduce off-target effects and be rapidly designed to target specific tissues, genetic sequences, or pathogens; the potential of our peptide-conjugated PMO ("PPMO") to be tailored to reach other organs beyond muscle; the potential of micro-dystrophin and Galgt2 to treat all or nearly all Duchenne muscular dystrophy ("DMD") patients regardless of mutation; and CRSPR/Cas9's potential to be used to fix stop codon mutations in the dystrophin gene so that dystrophin can be translated to a function protein;
- our belief that our highly differentiated, novel, proprietary and innovative RNA-targeted PMO-based platforms may represent a significant improvement over other RNA-targeted technologies;
- our belief that our PMO-based compounds could potentially be applied to treat a broad spectrum of diseases;
- our belief that golodirsen and casimersen will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping;
- the timely completion and satisfactory outcome of our post-marketing requirements and commitments, including verification of a clinical benefit for EXONDYS 51 in confirmatory trials;
- our ability to successfully expand the global footprint of eteplirsen in jurisdictions in which we have yet to obtain or do not have any near term ability or plans to obtain a full regulatory approval, including through obtaining an approval from the European Medicines Agency in the EU ("EMA"), establishing compliant and successful managed access programs ("MAP"), expanding our MAPs to include more countries over time, entering into any additional distribution, service and other contracts and building out the commercial, medical and other company infrastructure necessary to support the launch and support the distribution of eteplirsen in jurisdictions outside of the U.S.;
- the expectation that the European Commission will adopt the opinion of the Committee for Medicinal Products for Human Use ("CHMP") by year-end 2018;
- the potential acceptance of EXONDYS 51, and our product candidates if they receive regulatory approval, in the marketplace and the accuracy of our projections regarding the market size in each of the jurisdictions that we target;
- our ability to further secure long term supply of EXONDYS 51 and our product candidates, including our PPMO, to satisfy our planned commercial, MAP, named-patient program and clinical needs;
- our expectations regarding our ability to successfully conduct or accelerate research, development, pre-clinical, clinical and post-approval trials, and our expectations regarding the timing, design and results of such trials, including the potential consistency of data produced by these trials with prior results, as well as any new data and analyses relating to the safety profile and potential clinical benefits of EXONDYS 51 and our product candidates, including golodirsen, casimersen, PPMO and gene therapy-based product candidates;
- expected milestones and payments in connection with our agreements with Myonexus Therapeutics, Inc.("Myonexus"), Lacerta Therapeutics, Inc. ("Lacerta") and Lysogene S.A. ("Lysogene"), including the expectation to dose the first patients in the MYO-101 program and in the LYS-SAF302 pivotal trial in the fourth quarter of 2018;
- the impact of regulations and regulatory decisions and guidelines by the United States Food and Drug Administration ("FDA") and other regulatory agencies on our business, as well as the development of our product candidates and our

financial and contractual obligations;

- the expectation that there will be no material delay to the micro-dystrophin gene therapy program due to the clinical hold, which was lifter by the FDA;

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

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

Overview

We are a commercial-stage biopharmaceutical company focused on helping patients through the discovery and development of unique RNA-targeted therapeutics, gene therapy and other genetic medicine approaches for the treatment of rare neuromuscular diseases. Applying our proprietary, highly-differentiated and innovative RNA-targeted platform technologies, we are able to develop candidate therapies for a broad range of diseases and

disorders.

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

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

- EXONDYS 51, our first product, uses our PMO chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. EXONDYS 51 is designed to bind to exon 51 of dystrophin pre-messenger RNA ("mRNA"), resulting in exclusion, or "skipping", of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein.

We are in the process of conducting, starting or planning various EXONDYS 51 clinical trials, including studies that we need to conduct to comply with our post-marketing FDA requirements/commitments to verify and describe clinical benefit of EXONDYS 51.

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

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

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

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

- SRP-5051, one of our main product candidates, uses our next-generation chemistry platform, PPMO, and our exon-skipping technology to skip exon 51 of the dystrophin gene. SRP-5051, a peptide conjugated PMO, is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion, or "skipping", of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein.

In the fourth quarter of 2017, we received clearance from the FDA and commenced a first-in-human, single ascending dose study for our PPMO for the treatment of DMD in patients who are amenable to exon 51 skipping (SRP-5051). In addition to SRP-5051, our 2018 plans currently include IND-enabling pre-clinical work on additional PPMOs.

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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 and other rare neuromuscular diseases. These strategic partners include:

- Nationwide Children's Hospital. Our collaboration with Nationwide Children's Hospital include the advancement of their micro-dystrophin gene therapy program and their Galgt2 gene therapy program under exclusive license agreements. In the fourth quarter of 2017, the IND applications for both programs were cleared by the FDA, and two Phase 1/2a clinical trials in individuals with DMD were initiated. On October 3, 2018, Nationwide Children's Hospital presented positive updated results from its Phase 1/2a micro-dystrophin gene therapy clinical trial in the four individuals with DMD enrolled in the trial.
- Myonexus, which develops gene therapy programs for various forms of Limb-girdle muscular dystrophies ("LGMDs"). On May 3, 2018, we entered into an agreement with Myonexus that provides us with an exclusive option to acquire Myonexus by making an option exercise payment to Myonexus plus contingent payments, if earned.
- Lacerta, a gene therapy company that develops central nervous system ("CNS")-targeted treatments. On August 8, 2018, we entered into a license and option agreement with Lacerta, which provides us with a license to up to three new CNS-targeted gene therapy programs, including exclusive rights to Lacerta's gene therapy candidate for Pompe Disease and options to two additional candidates.
- Lysogene, a biopharmaceutical company specializing in gene therapy targeting CNS diseases. On October 15, 2018, we entered into a license and collaboration agreement with Lysogene for the development of a gene therapy, LYS-SAF302, to treat Mucopolysaccharidosis type IIIA ("MPS IIIA"). The first patient in the LYS-SAF302 pivotal trial is expected to be dosed in the fourth quarter of 2018. Under the agreement with Lysogene, we also have certain option rights to an additional CNS-targeted gene therapy candidate.
- Genethon, with whom we are collaborating on the advancement of their micro-dystrophin gene therapy program under a sponsored research and exclusive license option agreement.
- Duke University, with whom we are collaborating on the advancement of gene editing CRISPR/Cas9 technology for muscular dystrophy under a sponsored research and exclusive license option agreement that grants us rights to certain of Duke University's intellectual property for CRISPR/Cas9.
- Summit (Oxford) Ltd. ("Summit"), with whom we are collaborating under an exclusive license and collaboration agreement that grants us exclusive rights to Summit's utrophin modulator pipeline, including ezutromid, in Europe, Turkey and the Commonwealth of Independent States and an option to acquire rights in Latin America. On June 27, 2018, Summit announced that it decided to discontinue the development of ezutromid after reviewing the top-line results from its Phase 2 trial.

Our Proprietary Platform Technologies

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

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

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

We also collaborate with different partners to explore a gene therapy approach to DMD and other rare neuromuscular diseases. The programs in collaboration with Nationwide Children's Hospital and Genethon look to express a smaller but still functional version of dystrophin ("micro-dystrophin"). Micro-dystrophin is used because normal-sized dystrophin is too large to fit in an AAV. An additional program, also in collaboration with Nationwide Children's Hospital, aims to express the enzyme Galgt2 from an AAV vector. We believe that Galgt2 modifies the dystrophin associated protein complex and up-regulates utrophin (a protein significantly homologous to dystrophin) to protect muscle from damage in the absence of dystrophin. The micro-dystrophin and Galgt2 technologies have the potential to treat all or nearly all DMD patients regardless of mutation.

The most advanced of Myonexus' programs, MYO-101, is designed to transfer a gene that codes for and restores beta-sarcoglycan protein with the goal of restoring the dystrophin associated protein complex. It utilizes the AAVrh.74 vector system, the same vector used in the micro-dystrophin gene therapy program we are developing with Nationwide Children's Hospital.

The collaboration with Lacerta utilizes proprietary AAV capsid variants and a scalable vector manufacturing platform to develop treatments for CNS and lysosomal storage diseases. The lead candidate is a gene therapy approach with a novel AAV variant for treatment of Pompe Disease.

The program in collaboration with Lysogene focuses on the development of a gene therapy, LYS-SAF302, to treat MPS IIIA. LYS-SAF302 is an AAV-mediated gene therapy, the goal of which is to replace the faulty SGSH gene with a healthy copy of the gene. LYS-SAF302 employs the AAVrh10 virus, chosen for its ability to target the CNS. Proof-of-concept was established in MPS IIIA pre-clinical models demonstrating strong expression, broad distribution, and the ability of the compound to correct lysosomal storage defects by producing the missing enzyme.

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

Manufacturing

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

Cash, Cash Equivalents and Investments

As of September 30, 2018, we had approximately $793.9 million of cash, cash equivalents and investments, consisting of $209.7 million of cash and cash equivalents, $583.2 of short term investments, and $1.0 million of long-term restricted investment. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve months.

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

which we operate.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations is based upon our unaudited condensed consolidated financial statements included elsewhere in this report. The preparation of our unaudited condensed consolidated financial statements in accordance with accounting principles generally accepted in the United States requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities for the periods presented. Some of these judgments can be subjective and complex and, consequently, actual results may differ from these estimates. We believe that the estimates and judgments upon which we rely are reasonable based upon historical experience and information available to us at the time that we make these estimates and judgments. To the extent there are material differences between these estimates and actual results, our unaudited condensed consolidated financial statements will be affected. Although we believe that our judgments and estimates are appropriate, actual results may differ from these estimates. We believe the following accounting policies to be the most critical to the judgements and estimates used in the preparation of our unaudited condensed consolidated financial statements:

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

Except as described in Note 2 to our accompanying condensed consolidated financial statements with respect to changes in our revenue recognition policy related to our adoption of the requirements of ASC 606, there have been no changes to our critical accounting policies and significant estimates as detailed in our Annual Report on Form 10-K for the year ended December 31, 2017.

Results of Operations for the Three and Nine Months Ended September 30, 2018 and 2017

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

	For the Three Months Ended September 30,			
	2018	2017	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$78,486	$45,954	$32,532	71 %
Total revenues	78,486	45,954	32,532	71 %
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	8,741	3,078	5,663	NM
Research and development	86,584	34,239	52,345	153 %
Selling, general and administrative	53,044	28,176	24,868	88 %
EXONDYS 51 litigation and license charges	—	25,588	(25,588)	(100)%
Amortization of in-licensed rights	216	780	(564)	NM
Total cost and expenses	148,585	91,861	56,724	62 %
Operating loss	(70,099)	(45,907)	(24,192)	53 %
Other (loss) income:				
Interest (expense) income and other, net	(6,968)	184	(7,152)	NM
Loss before income tax (benefit) expense	(77,067)	(45,723)	(31,344)	69 %
Income tax (benefit) expense	(674)	2,011	(2,685)	NM
Net loss	(76,393)	$(47,734)	$(28,659)	60 %
Net loss per share - basic and diluted	$(1.15)	$(0.78)	$(0.38)	49 %

	For the Nine Months Ended September 30,			
	2018	2017	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$216,619	$97,307	$119,312	123 %
Total revenues	216,619	97,307	119,312	123 %
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	21,058	3,807	17,251	NM
Research and development	255,636	122,266	133,370	109 %
Selling, general and administrative	143,541	90,461	53,080	59 %
EXONDYS 51 litigation and license charges	—	28,427	(28,427)	(100)%
Amortization of in-licensed rights	649	837	(188)	(22)%
Total cost and expenses	420,884	245,798	175,086	71 %
Operating loss	(204,265)	(148,491)	(55,774)	38 %
Other (loss) income:				

Gain from sale of Priority Review Voucher	—	125,000	(125,000)	(100)%
Interest (expense) income and other, net	(16,671)	703	(17,374)	NM
Loss before income tax expense	(220,936)	(22,788)	(198,148)	NM
Income tax expense	87	3,902	(3,815)	(98)%
Net loss	(221,023)	$(26,690)	$(194,333)	NM
Net loss per share - basic and diluted	$(3.38)	$(0.47)	$(2.91)	NM

* NM = Not Meaningful

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Revenues

Revenues from product sales are recorded at the net sales price (transaction price), which includes estimates of variable consideration for which reserves are established and which result from Medicaid rebates, governmental chargebacks including Public Health Services chargebacks, prompt pay discounts, co-pay assistance and distribution fees. These reserves are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable (if no payments are required of us) or a current liability (if a payment is required of us). 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. The amount of variable consideration that is included in the transaction price may be constrained, and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received or paid may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known. Net product revenues for EXONDYS 51 for the three and nine months ended September 30, 2018 increased by $32.5 million and $119.3 million compared with the three and nine months ended September 30, 2017, respectively. These increases for both the three and nine months ended September 30, 2018 primarily reflect increasing demand for EXONDYS 51 in the U.S.

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

Our cost of sales (excluding amortization of in-licensed rights) primarily consists of royalty payments to BioMarin Pharmaceutical, Inc. (“BioMarin”) as a result of the execution of the settlement and licenses agreements in July 2017, inventory costs that relate to sales of EXONDYS 51 following our commercial launch in the U.S., and overhead costs. Prior to receiving regulatory approval for EXONDYS 51 from the FDA in September 2016, we expensed such manufacturing and material costs as research and development expenses. For EXONDYS 51 sold in the three and nine months ended September 30, 2018 and 2017, certain manufacturing costs incurred had previously been expensed as research and development expenses, as such costs were incurred prior to the FDA approval of EXONDYS 51. If product related costs had not previously been expensed as research and development expenses prior to receiving FDA approval, the incremental cost to produce the EXONDYS 51 sold would have been approximately $9.5 million and $5.4 million for the nine months ended September 30, 2018 and 2017, respectively.

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

	For the Three Months Ended September 30,				
	2018	2017	Change $	Change %	
	(in thousands)				
Royalty payments to BioMarin	$4,001	$2,289	1,712	75	%
Inventory costs related to EXONDYS 51 sold	2,676	211	2,465	NM	
Overhead costs	1,640	516	1,124	218	%
Other inventory costs	424	62	362	NM	
Total cost of sales	$8,741	$3,078	5,663	NM	

	For the Nine Months Ended September 30,

	2018	2017	Change $	Change %	
	(in thousands)				
Royalty payments to BioMarin	$10,813	$2,289	8,524	NM	
Inventory costs related to EXONDYS 51 sold	6,215	314	5,901	NM	
Overhead costs	3,604	1,113	2,491	224	%
Other inventory costs	426	91	335	NM	
Total cost of sales	$21,058	$3,807	17,251	NM	

* NM = Not Meaningful

The cost of sales for the three and nine months ended September 30, 2018, increased by $5.7 million and $17.3 million compared with the same periods in 2017. The increase for both periods primarily reflects royalty payments to BioMarin as a result of the execution of the settlement and license agreements with BioMarin in July 2017 as well as increasing demand for EXONDYS 51.

Research and Development Expenses

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

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

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

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

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

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

	For the Three Months Ended September 30,				
	2018	2017	Change $	Change %	
	(in thousands)				
Up-front and milestone payments	$18,000	$—	$18,000	NM	
Eteplirsen (exon 51)	8,738	8,337	401	5	%
PPMO platform	7,665	2,452	5,213	100	%
Casimersen (exon 45)	6,004	5,279	725	14	%
Golodirsen (exon 53)	6,193	4,569	1,624	36	%
Collaboration cost-sharing	1,570	—	1,570	NM	
Other projects	2,548	286	2,262	NM	

Internal research and development expenses	35,866	13,316	22,550	169	%
Total research and development expenses	$86,584	$34,239	$52,345	153	%

	For the Nine Months Ended				
	September 30,				
	2018	2017	Change	Change	
	(in thousands)		$	%	
Up-front and milestone payments	$78,000	$22,000	$56,000	255	%
Eteplirsen (exon 51)	24,961	27,821	(2,860)	(10)	%
Casimersen (exon 45)	21,342	13,266	8,076	61	%
Golodirsen (exon 53)	20,598	12,709	7,889	62	%
PPMO platform	16,461	6,300	10,161	161	%
Collaboration cost-sharing	7,632	—	7,632	35	%
Other projects	3,952	1,191	2,761	232	%
Internal research and development expenses	82,690	38,979	43,711	112	%
Total research and development expenses	$255,636	$122,266	$133,370	109	%

The Company has revised the presentation as well as certain captions in the research and development expenses by project table presented above. “PPMO platform” of $2.5 million and $6.3 million for the three and nine months ended September 30, 2017 was reclassified from “other projects” and presented separately in the table to conform to current year presentation.

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

	For the Three Months Ended September 30,			
	2018	2017	Change $	Change %
	(in thousands)			
Clinical and manufacturing expenses	$29,154	$16,381	$12,773	78 %
Up-front and milestone payments	18,000	—	18,000	NM
Compensation and other personnel expenses	14,061	5,957	8,104	136 %
Professional services	3,627	2,860	767	27 %
Pre-clinical expenses	7,141	3,304	3,837	116 %
Stock-based compensation	3,260	1,812	1,448	80 %
Facility-related expenses	4,852	2,269	2,583	114 %
Collaboration cost-sharing	1,570	—	1,570	NM
Research and other	4,919	1,656	3,263	197 %
Total research and development expenses	$86,584	$34,239	$52,345	153 %

Research and development expenses for the three months ended September 30, 2018 increased by $52.3 million, or 153%, compared with the three months ended September 30, 2017. The increase was primarily driven by the following:

- $12.8 million increase in clinical and manufacturing expenses primarily due to increased patient enrollment in our ongoing ESSENCE trial as well as a ramp-up of manufacturing activities for golodirsen, our microdystrophin program, and our PPMO platform. These increases were partially offset by a ramp-down of clinical trials in eteplirsen primarily because the PROMOVI trial has been fully enrolled;
- $18.0 million increase in up-front and milestone payments driven by $10.0 million milestone payment to Myonexus as a result of the achievement of one of the development milestones as well as $8.0 million related to the purchase of a license to develop, manufacture and commercialize a pre-clinical Pompe product candidate under the License Agreement with Lacerta.
- $8.1 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $0.8 million increase in professional services primarily due to continuing accelerated company growth as a result of expansion of our research and development pipeline;
- $3.8 million increase in pre-clinical expenses primarily due to the continuing ramp-up of toxicology studies in our PPMO platform;
- $1.4 million in stock-based compensation expense primarily driven by increases in headcount and stock price;
- $2.6 million increase in facility-related expenses due to our continuing expansion efforts;
- $1.6 million increase in collaboration cost sharing with Summit on its Utrophin platform; and
-

$2.6 million increase in sponsored research with institutions such as Duke University and Nationwide Children’s Hospital

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	For the Nine Months Ended				
	September 30,				
	2018	2017	Change	Change	
	(in thousands)		$	%	
Up-front and milestone payments	$78,000	$22,000	$56,000	255	%
Clinical and manufacturing expenses	75,630	50,650	24,980	49	%
Compensation and other personnel expenses	34,471	17,738	16,733	94	%
Professional services	12,108	7,327	4,781	65	%
Pre-clinical expenses	15,223	7,326	7,897	108	%
Stock-based compensation	10,349	5,881	4,468	76	%
Facility-related expenses	11,292	6,636	4,656	70	%
Collaboration cost-sharing	7,632	—	7,632	NM	
Research and other	10,931	4,708	6,223	132	%
Total research and development expenses	$255,636	$122,266	$133,370	109	%

Research and development expenses for the nine months ended September 30, 2018 increased by $133.4 million, or 109%, compared with the nine months ended September 30, 2017. The increase was primarily driven by the following:

- $56.0 million increase in up-front and milestone payments due to an up-front payment of $60.0 million to Myonexus upon execution of the Warrant Agreement in May 2018, a milestone payment of $10.0 million to Myonexus upon achievement of one of the development milestones in September 2018 and $8.0 million related to the purchase of a license to develop, manufacture and commercialize a pre-clinical Pompe product candidate under the License Agreement with Lacerta in August 2018. In May 2017, we made a milestone payment of $22.0 million to Summit as the milestone of the last patient dosed in the safety arm cohort to the PhaseOut DMD study was achieved;
- $25.0 million increase in clinical and manufacturing expenses primarily due to increased patient enrollment in our ongoing ESSENCE trial as well as a ramp-up of manufacturing activities for golodirsen, casimersen, our microdystrophin program, and our PPMO platform. These increases were partially offset by a ramp-down of clinical trials in eteplirsen primarily because the PROMOVI trial has been fully enrolled;
- $16.7 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $4.8 million increase in professional services primarily due to continuing accelerated company growth as a result of expansion of our research and development pipeline;
- $7.9 million increase in pre-clinical expenses primarily due to the continuing ramp-up of toxicology studies in our PPMO platform as well as golodirsen and casimersen;
- $4.5 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price, as well as achievement of a milestone related to the September 2016 restricted stock awards with a performance condition;
- $4.7 million increase in facility-related expenses due to our continuing expansion efforts;
- $7.6 million increase in collaboration cost sharing with Summit on its Utrophin platform; and
- $4.0 million increase in sponsored research with institutions such as Duke University, Genethon and Nationwide Children’s Hospital.

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,				
	2018	2017	Change $	Change %	
	(in thousands)				
Professional services	$20,547	10,713	$9,834	92	%
Compensation and other personnel expenses	18,627	9,204	9,423	102	%
Stock-based compensation	8,224	5,178	3,046	59	%
Facility-related expenses	2,910	1,203	1,707	142	%
Former CEO severance	—	137	(137)	(100)	%
Other	2,736	1,741	995	57	%
Total selling, general and administrative expenses	$53,044	$28,176	$24,868	88	%

Selling, general and administrative expenses for the three months ended September 30, 2018 increased by $24.9 million, or 88%, compared with the three months ended September 30, 2017. This was primarily driven by the following:

- $9.8 million increase in professional services primarily due to continuing global expansion;
- $9.4 million increase in compensation and other personnel expenses primarily due to an increase in headcount;
- $3.0 million increase in stock-based compensation primarily due to increases in headcount and stock price; and
- $1.7 million increase in facility-related expense primarily due to continuing global expansion.

	For the Nine Months Ended September 30,				
	2018	2017	Change $	Change %	
	(in thousands)				
Professional services	$53,456	$31,642	$21,814	69	%
Compensation and other personnel expenses	49,783	26,718	23,065	86	%
Stock-based compensation	26,940	15,087	11,853	79	%
Facility-related expenses	7,236	4,355	2,881	66	%
Former CEO severance	—	3,537	(3,537)	(100)	%
Restructuring expenses	(2,222)	2,589	(4,811)	(186)	%
Other	8,348	6,533	1,815	28	%

Total selling, general and administrative expenses	$143,541	$90,461	$53,080	59	%

Selling, general and administrative expenses for the nine months ended September 30, 2018 increased by $53.1 million, or 59%, compared with the nine months ended September 30, 2017. This was primarily driven by the following:

- $21.8 million increase in professional services primarily due to continuing global expansion;
- $23.1 million increase in compensation and other personnel expenses primarily due to an increase in headcount;
- $11.9 million increase in stock-based compensation primarily due to increases in headcount and stock price, the achievement of a milestone related to the September 2016 restricted stock awards with performance conditions as well as the impact of revised forfeiture rate assumption for officers and members of our Board of Directors;
- $2.9 million increase in facility-related expense primarily due to continuing global expansion;
- $3.5 million decrease in severance expense as a result of the termination of our former CEO in June 2017; and
- $4.8 million decrease in restructuring expenses due to the relief of cease-use liabilities as a result of the termination of the rental agreement for our Corvallis facility.

EXONDYS 51 litigation and license charges

As a result of the execution of the settlement and license agreements with BioMarin in July 2017, we recognized EXONDYS 51 litigation and license charges of $25.6 million and $28.4 million during the three and nine month ended September 30, 2017, respectively. There was no such a transaction in 2018.

Amortization of In-licensed Rights

Amortization of in-license rights relate to the two agreements we entered into with BioMarin and University of Western Australia ("UWA") in July 2017 and April 2011, respectively. We recorded an in-licensed right asset of $6.6 million as a result of a settlement and license agreement with BioMarin. Additionally, following the first sale of EXONDYS 51 in September 2016, we recorded an in-licensed right asset of $1.0 million related to a license agreement with UWA. Both in-licensed rights are being amortized on a straight-line basis over the life of the patent from the first commercial sale of EXONDYS 51. For the three and nine months ended September 30, 2018, we recorded amortization of in-licensed rights of approximately $0.2 million and $0.6 million, respectively. 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. There was no such a transaction in 2018.

Interest (expense) income and other, net

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

For the three and nine months ended September 30, 2018, interest expense and other, net was approximately $7.0 million and $16.7 million, respectively. 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. The unfavorable changes primarily reflected the interest expense accrued on our debt facilities entered into in November 2017 partially offset by interest income from higher balances of cash, cash equivalents and investments.

Income tax (benefit) expense

Income tax (benefit) expense for the three and nine months ended September 30, 2018 was approximately ($0.7) million and $0.1 million, respectively, which related to state taxes. Income tax (benefit) expense for the three and nine months ended September 30, 2017 was approximately $2.0 million and $3.9 million, respectively, which related to the alternative minimum tax related the gain from the sale of the PRV.

Liquidity and Capital Resources

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

	As of September 30, 2018	As of December 31, 2017	Change $	Change %
	(in thousands)			
Financial assets:				
Cash and cash equivalents	$209,702	$599,691	$(389,989)	(65)%
Short-term investments	583,158	479,369	103,789	22 %
Long-term investment	—	9,980	(9,980)	(100)%
Restricted investment	1,001	784	217	28 %
Total cash, cash equivalents and investments	$793,861	$1,089,824	$(295,963)	(27)%
Borrowings:				
Long-term debt	$—	$30,410	$(30,410)	(100)%
Convertible debt	415,446	400,641	14,805	4 %
Total borrowings	$415,446	$431,051	$(15,605)	(4)%
Working capital				
Current assets	$1,012,077	$1,228,644	$(216,567)	(18)%
Current liabilities	$114,393	88,332	26,061	30 %
Total working capital	$897,684	$1,140,312	$(242,628)	(21)%

For the period ended September 30, 2018, our principal source of liquidity was derived from proceeds from product sales of EXONDYS 51 and equity and debt financings. For the period ended December 31, 2017, our principal source of liquidity was derived from proceeds from the sale of the PRV, equity and debt financings and product sales of EXONDYS 51. Our principal uses of cash are research and development expenses, selling, general and administrative expenses, investments, capital expenditures, business development transactions and other working capital requirements.

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

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

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

Cash Flows

	For the Nine Months Ended September 30,			
	2018	2017	Change $	Change %
	(in thousands)			
Cash provided by (used in)				
Operating activities	$(266,240)	$(200,938)	$(65,302)	32%
Investing activities	$(132,752)	314,880	(447,632)	(142)%
Financing activities	$8,867	381,269	(372,402)	(98)%
(Decrease) increase in cash and cash equivalents	$(390,125)	$495,211	$(885,336)	(179)%

*NM = Not Meaningful

Operating Activities. Cash used in operating activities increased by $65.3 million for the nine months ended September 30, 2018 compared with the nine months ended September 30, 2017. This was primarily driven by an increase of $69.3 million in net loss excluding the gain from sale of the PRV due to an increase in product sales for EXONDYS 51 partially offset by increases in research and development expenses, including $78.0 million up-front and milestone payments, and selling, general and administrative expenses, and unfavorable changes of $25.1 million in operating assets and liabilities primarily due to timing of certain payments, including $35.0 million payments to Brammer Bio MA, LLC, offset by an increase of $26.9 million in non-cash adjustments.

Investing Activities. The cash used in investing activities for the nine months ended September 30, 2018 was $132.8 million and the cash provided by investing activities for the nine months ended September 30, 2017 was $314.9 million. The unfavorable change was driven by increases of $551.0 million in purchase of available-for-sale securities and $32.9 million in property and equipment as well as proceeds of $125.0 million from sale of the PRV and $10.7 million in maturity of restricted investment in March 2017. These were partially offset by an increase of $266.4 million from the maturity and sales of available-for-sale securities and a decrease of $6.0 million in purchase of intangible assets.

Financing Activities. Cash provided by financing activities decreased by $372.4 million for the nine months ended September 30, 2018 compared with the nine months ended September 30, 2017. This was primarily driven by decreases of $354.0 million in net proceeds from sales of common stock and an increase of $213.8 million in repayment of our debt facilities and debt extinguishment costs. These were partially offset by an increase of $31.3 million in proceeds from exercise of stock options and purchase of stock under the Employee Stock Purchase Program and an increase of $164.1 in proceeds from our debt facilities.

Off-Balance Sheet Arrangements

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

Contractual Payment Obligations

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

	Payment Due by Period				
	Total	Less Than 1 Year	1 - 3 Years	3 - 5 Years	More than 5 Years
	(in thousands)				
Convertible debt (1)	622,369	8,550	17,100	17,100	579,619
Lease obligations	60,042	7,460	15,824	18,006	18,752
Manufacturing obligations (2)	402,123	107,487	86,636	78,000	130,000
Total contractual obligations and contingencies	$1,084,534	$123,497	$119,560	$113,106	$728,371

(1) Interest is included.

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

Milestone Obligations

For product candidates that are currently in various research and development stages, we may be obligated to make up to $289.8 million of future development, regulatory, and commercial, and up-front royalty milestone payments associated with our collaboration and license agreements. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory or commercial milestones. Because the achievement of these milestones is not probable and payment is not required as of September 30, 2018, such contingencies have not been recorded in our unaudited condensed consolidated financial statements. Amounts related to contingent milestone payments are not yet considered contractual obligations as they are contingent on the successful achievement of certain development, regulatory approval and sales milestones.

Recent Accounting Pronouncements

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

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our current investment policy is to maintain a diversified investment portfolio consisting of money market investments, government and government agency bonds and high-grade corporate bonds with maturities of three years or less. Our cash is deposited in and invested through highly rated financial institutions in North America. As of September 30, 2018, we had approximately $793.9 million of cash, cash equivalents and investments, comprised of $209.7 million of cash and cash equivalents, $583.2 million of short-term investments and $1.0 million long-term restricted investment. The fair value of cash equivalents and short-term investments is subject to change as a result of potential changes in market interest rates. The potential change in fair value for interest rate sensitive instruments has been assessed on a hypothetical 10 basis point adverse movement across all maturities. As of September 30, 2018, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of approximately $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, 2018, under the supervision and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of our disclosure controls and procedures pursuant to paragraph (b) of Rules 13a-15 and 15d-15 under the Securities Exchange Act of 1934 (the “Exchange Act”). The purpose of this evaluation was to determine whether as of the evaluation date our disclosure controls and procedures were effective to provide reasonable assurance that the information we are required to disclose in our filings with the SEC under the Exchange Act (i) is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms and (ii) is accumulated and communicated to our management, including our Chief Executive Officer and our Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of September 30, 2018, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

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

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PART II — OTHER INFORMATION

Item 1. Legal Proceedings

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

Item 1A. Risk Factors.

Factors That Could Affect Future Results

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

Risks Related to Our Business

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

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

- the effectiveness of our sales, managed markets, marketing efforts and support for EXONDYS 51;
- the consistency of any new data we collect and analyses we conduct with prior results, whether they support a favorable safety and efficacy profile of EXONDYS 51 and any potential impact on our FDA accelerated approval status and/or FDA package insert for EXONDYS 51;
- the effectiveness of our ongoing EXONDYS 51 commercialization activities, including negotiating and entering into any additional commercial, supply and distribution contracts, scaling up manufacturing and hiring any additional personnel as needed to support commercial efforts;
- our ability to comply with FDA post-marketing requirements and commitments, including through successfully conducting additional studies that confirm clinical efficacy and safety of EXONDYS 51 and acceptance of the same by the FDA and medical community since continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials;
- the occurrence of any side effects, adverse reactions or misuse, or any unfavorable publicity in these areas;
- the cost-effectiveness of EXONDYS 51 and whether we can consistently manufacture it in commercial quantities and at acceptable costs;
- the rate and consistency with which EXONDYS 51 is prescribed by physicians, which depends on physicians' views on the safety and efficacy of EXONDYS 51;
- our ability to secure and maintain adequate reimbursement for EXONDYS 51, including during re-authorizations processes that may be required for patients who initially obtained coverage by third parties, including government payors, managed care organizations and private health insurers;
- our ability to obtain and maintain patent protection for EXONDYS 51, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing on the proprietary rights of third

parties;
- the development or commercialization of competing products or therapies for the treatment of DMD, or its symptoms, and the existence of competing clinical trials;
- our ability to increase awareness of the importance of genetic testing and knowing/understanding DMD mutations, and identifying and addressing procedural barriers to obtaining therapy;
- our ability to remain compliant with laws and regulations that apply to us and our commercial activities;

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

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

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

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

Although we contracted with third party distributors to distribute eteplirsen in certain countries outside the U.S. on a named patient basis, and initiated a limited launch of an ex-U.S. eteplirsen MAP, which we plan to expand to other jurisdictions in the future, and although we continue to pursue regulatory approval of eteplirsen in certain targeted jurisdictions, such as the EU and Israel, we may not be successful in expanding access to eteplirsen nor produce any significant revenues from eteplirsen sales outside of the U.S. For example, healthcare providers in MAP jurisdictions may not be convinced that their patients can benefit from eteplirsen or may prefer to wait until such time as eteplirsen is approved by a regulatory authority in their country before prescribing eteplirsen. Even if a healthcare provider is interested in obtaining access to eteplirsen for its patient through the MAP, the patient will not be able to obtain access to eteplirsen if payment for the drug is not secured. Additionally, we may not be able to obtain regulatory approval in the jurisdictions we have targeted, such as the EU, if our product approval applications, data packages submitted to regulatory authorities, and any additional data and analyses we submit in response to requests and concerns from regulatory authorities, do not support or convince regulatory authorities of the safety and efficacy of eteplirsen. If we fail to obtain regulatory approvals, our ability to make revenues from eteplirsen sales outside of the U.S. will be limited. In addition, failure to obtain approval in one country or area may affect sales under the MAP in other countries or areas. Even if we are successful in obtaining regulatory approval of eteplirsen outside of the U.S., our revenue earning capacity will depend on commercial and medical infrastructure, pricing and reimbursement negotiations and decisions with third party payors, including government payors. On September 21, 2018, we announced that the CHMP of the EMA has confirmed its May 31, 2018 negative opinion for a Conditional Marketing Application for eteplirsen. See also "— Even though EXONDYS 51 has been approved for marketing in the U.S., we may not receive approval to commercialize EXONDYS 51 outside of the U.S."

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

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

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

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

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

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

For example, we were notified by the Research Institute at Nationwide Children’s Hospital (the “Research Institute”) that they received a letter from the FDA on July 24, 2018, stating that their Phase 1/2a DMD micro-dystrophin gene therapy trial had been placed on clinical hold due to the presence of a trace amount of DNA fragment in research-grade third-party supplied plasmid (the “Clinical Hold”). The Research Institute, working with us, developed an action plan with immediate plans to submit for review by the FDA, which included the use of GMP-s plasmid for the program. On September 24, 2018, we announced that the FDA had lifted the Clinical Hold and that we do not anticipate any material delay to this program.

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

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

We have, through our third party manufacturers, produced or are in the process of producing supply of our product candidates and EXONDYS 51, respectively, based on our current understanding of market demands and our anticipated needs for our research and development efforts, clinical trials, MAPs and commercial sales. In light of the limited number of third parties with the expertise to produce EXONDYS 51 and our product candidates, the lead time needed to manufacture them, and the availability of underlying materials, we may not be able to, in a timely manner or at all, establish or maintain sufficient commercial and other manufacturing arrangements on the commercially

reasonable terms necessary to provide adequate supply of EXONDYS 51 and our other product candidates to meet demands that meet or exceed our projected needs. Furthermore, we may not be able to obtain the significant financial capital that may be required in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for EXONDYS 51 and our product candidates, such parties may not comply with the terms and timelines they have agreed to for various reasons, some of which may be out of their or our control, which could impact our ability to execute our business plans on expected or required timelines in connection with the commercialization of EXONDYS 51 and the continued development of our product candidates, including our follow-on exon-skipping product candidates, PPMO and gene therapy-based product candidates. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and /or substantial termination penalties, which could have a material adverse effect on our business prior to and after commercialization.

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

Our contract manufacturers are required to produce our materials, APIs and drug products under cGMP. We and our contract manufacturers are subject to periodic inspections by the FDA, EMA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations. While we work diligently with all contract manufacturers to maintain full compliance, we do not have direct control over a third party manufacturer's compliance with these regulations and requirements. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of EXONDYS 51 and our product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively. The failure to achieve and maintain compliance with cGMP and other applicable government regulations, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in product recalls, clinical holds, delayed or withheld approvals, patient injury or death. This risk is particularly heightened as we optimize manufacturing for our product candidates, including golodirsen, casimersen, and novel programs such as PPMO and gene therapy. For example, following the imposition of the Clinical Hold, the Research Institute, working with us, developed an action plan with immediate plans to submit for review by the FDA, which included the use of GMP-s plasmid for the Nationwide Children's Hospital's Phase 1/2a DMD micro-dystrophin gene therapy trial. On September 24, 2018, we announced that the FDA had lifted the Clinical Hold. If our contract manufacturers fail to adhere to applicable cGMP and other applicable government regulations, or experience manufacturing problems, we will suffer significant consequences, including product seizures or recalls, postponement or cancellation of clinical trials, loss or delay of product approval, fines and sanctions, loss of revenue, termination of the development of a product candidate, reputational damage, shipment delays, inventory shortages, inventory write-offs and other product-related charges and increased manufacturing costs. If we experience any of these results, the success of our commercialization of EXONDYS 51 and/or our development efforts for our product candidates, including golodirsen, casimersen and novel programs such as PPMO and gene therapy, could be significantly delayed, fail or otherwise be negatively impacted.

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

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

manufacturing processes and analytical methods or to demonstrate adequate purity, stability or comparability, the commercial availability of EXONDYS 51 and the continued development and/or regulatory approval of our product candidates, including PPMO and gene therapy-based product candidates, may be delayed or otherwise negatively impacted, which could significantly harm our business.

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

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

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

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

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

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

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

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

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

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

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

- unforeseen costs and expenses associated with maintaining and further developing an independent sales and marketing organization.

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

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

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

We expect that private insurers will continue to consider the efficacy, cost-effectiveness and safety of EXONDYS 51, including any new data and analyses that we are able to collect and make available in a compliant manner, in determining whether to approve reimbursement for EXONDYS 51 and at what levels. If any new data and information we collect is not favorable, third party insurers may make coverage decisions that negatively impact sales of EXONDYS 51. We continue to have discussions with payors, some of which may eventually deny coverage. We may not receive approval for reimbursement of EXONDYS 51 from additional insurers on a satisfactory rate or basis, in which case our business would be materially adversely affected. In addition, obtaining these approvals can be a time consuming and expensive process. Our business would be materially adversely affected if we are not able to maintain favorable coverage decisions and/or fail to receive additional favorable coverage decisions from third party insurers, in particular during re-authorization processes for patients that have already initiated therapy. Our business could also be adversely affected if insurers, including managed care organizations, the Medicare or Medicaid programs or other reimbursing bodies or payors limit the indications for which EXONDYS 51 will be reimbursed or fail to recognize accelerated approval and surrogate endpoints as clinically meaningful.

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

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

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

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

The U.S. government and individual states have aggressively pursued healthcare reform, as evidenced by the passing of the Healthcare Reform Act and the ongoing efforts to modify or repeal that legislation. The Healthcare Reform Act substantially changed the way healthcare is financed by both governmental and private insurers and contains a number of provisions that affect coverage and reimbursement of drug products and/or that could potentially reduce the

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

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

- controls on government funded reimbursement for drugs;
- caps or mandatory discounts under certain government sponsored programs;
- controls on healthcare providers;
- challenges to the pricing of drugs or limits or prohibitions on reimbursement for specific products through other means;
- reform of drug importation laws;
- delegation of decision making to state Medicaid agencies and waiver of reimbursement requirements;
- expansion of use of managed care systems in which healthcare providers contract to provide comprehensive healthcare for a fixed cost per person; and
- prohibition on direct-to-consumer advertising or drug marketing practices.

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

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

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

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

Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials. These post-approval requirements and commitments may not be feasible and/or could impose significant burdens and costs on us; could negatively impact our development, manufacturing and supply of EXONDYS 51; and could negatively impact our financial results. Failure to meet post-approval commitments and requirements, including completion of enrollment and in particular, any failure to obtain positive safety and efficacy data from our ongoing and planned EXONDYS 51 studies, would lead to negative regulatory action from the FDA and/or withdrawal of regulatory approval of EXONDYS 51. In addition, on September 21, 2018, we announced that the CHMP of the EMA has confirmed its May 31, 2018 negative opinion for a Conditional Marketing Application for eteplirsen. Such negative opinion could negatively impact our approval status in the U.S.

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

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

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

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

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

In November 2016, we submitted a marketing authorization application ("MAA") for eteplirsen to the EMA and the application was validated in December 2016. As we announced on June 1, 2018, the CHMP of the EMA adopted a negative opinion for eteplirsen. On September 21, 2018, we announced that the CHMP of the EMA has confirmed its

negative opinion for eteplirsen. We expect the European Commission to adopt the CHMP opinion by year-end 2018.

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

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

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

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

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

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

impacted. The degree of market acceptance of EXONDYS 51 depends on a number of factors, including:

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

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

The patient population suffering from DMD, LGMDs, Pompe Disease and MPS IIIA 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, LGMD, Pompe Disease and MPS IIIA are rare, fatal genetic neuromuscular disorders. DMD affects an estimated one in approximately every 3,500 to 5,000 males born worldwide, of which up to 13% are estimated to be amenable to exon-51 skipping. LGMDs as a class affect an estimated range of approximately one in every 14,500 to one in every 123,000 individuals. Pompe Disease affects an estimated one in approximately every 40,000 individuals. MPS IIIA affects approximately 1 in 100,000 newborns. Our estimates of the size of these patient populations are 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, LGMD, Pompe Disease and MPS IIIA, our assessment of the market may be inaccurate, making it difficult or impossible for us to meet our revenue goals, or to obtain and maintain profitability. The small population of DMD, LGMD, Pompe Disease and MPS IIIA patients may also delay patients' recruitment for our clinical trials, especially in light of competing clinical trials.

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

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

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

As discussed above, we are not guaranteed to receive or maintain orphan status for our current or future product candidates, and if our product candidates that are granted orphan status were to lose their status as orphan drugs or the marketing exclusivity provided for them in the U.S. or the EU, our business and operations could be adversely affected. While orphan status for any of our products, if granted or maintained, would provide market exclusivity in the U.S. and the EU for the time periods specified above upon approval, we would not be able to exclude other companies from obtaining regulatory approval of products using the same active ingredient for the same indication beyond the exclusivity period applicable to our product on the basis of orphan drug status. In addition, we cannot

guarantee that another company will not receive approval to market a product candidate that is granted orphan drug status in the U.S. or the EU for the same drug and orphan indication as any of our product candidates for which we plan to file an NDA or MAA. If that were to happen, any pending NDA or MAA for our product candidate for that indication may not be approved until the competing company's period of exclusivity has expired in the U.S. or the EU, as applicable.

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

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

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

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

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

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

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

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

Nationwide Children's Hospital, with whom we are collaborating, initiated Phase 1/2a clinical trials for their micro-dystrophin gene therapy program and their Galgt2 gene therapy program. On October 3, 2018, Nationwide Children's Hospital presented positive updated results from its Phase 1/2a micro-dystrophin gene therapy clinical trial in the four individuals with DMD enrolled in the trial. In addition, Myonexus, with whom we entered into a warrant to purchase common stock of Myonexus, expects to dose the first patient in the MYO-101 program by the end of 2018. The MYO-101 program, as well as four other, less advanced, Myonexus programs, aim to develop gene therapy-based treatments for various forms of LGMD. Furthermore, Lysogene, with whom we collaborate on developing a gene therapy, LYS-SAF302, to treat MPS IIIA, expects to dose the first patient in the LYS-SAF302 pivotal trial in the fourth quarter of 2018.

The remainder of our product candidates are in discovery or early stages of development. These product candidates will require significant further development, financial resources and personnel to develop into commercially viable products and obtain regulatory approval, if at all. Given the FDA approval of EXONDYS 51, we expect that much of our effort and many of our expenditures over the next several years will be devoted to clinical development and regulatory activities associated with EXONDYS 51 and other exon-skipping candidates as part of our larger follow-on exon strategy in DMD, our other disease candidates, our proprietary chemistry, and other potential therapeutic areas that provide long-term market opportunities. We may be delayed, restricted, or unable to further develop our active and other product candidates or successfully obtain approvals needed to market them. Although EXONDYS 51 was approved under accelerated approval by the FDA in the U.S., we may not be able to obtain an approval of EXONDYS 51 in the EU.

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

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

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

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive pre-clinical and clinical trials that the product candidate is safe and effective in humans. Ongoing and future pre-clinical and clinical trials of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. For example, although the pre-clinical data for PPMO collected to date is promising, the additional data we collect, including in the clinic, may not be consistent with the pre-clinical data or show a safe benefit that warrants further development or pursuit of a regulatory approval for PPMO product candidates. Furthermore, success in pre-clinical and early clinical trials does not ensure that the subsequent trials will be successful, nor does it predict final results of a confirmatory trial. For example, on October 3, 2018, Nationwide Children's Hospital presented positive updated results from its Phase 1/2a micro-dystrophin gene

therapy clinical trial in the four individuals with DMD enrolled in the trial. The preliminary data is based on a small patient sample and reported before completion of the trial and therefore may not be predictive of future results. In addition, we cannot assure that the results of additional preliminary data or data from the completed trial or any future trial will yield results that are consistent with the preliminary data presented, that we will be able to demonstrate the safety and efficacy of AAVrh74.MHCK7.micro-Dystrophin, that later trial results will support further development, or even if such later results are favorable, that we will be able to successfully complete the development of, obtain accelerated, conditional or standard regulatory approval for, or successfully commercialize AAVrh74.MHCK7.micro-Dystrophin. Similarly, we cannot provide assurances that data from our studies with respect to EXONDYS 51, golodirsen, casimersen and gene therapy-based product candidates will be positive and consistent through the study periods or that the interpretation by regulators, such as the FDA or EMA, of the data we collect for our product or product candidates will be consistent with our interpretations.

In addition, different methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters may yield different statistical results. Even if we believe the data collected from clinical trials of our product candidates are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities. Pre-clinical and clinical data can be interpreted in different ways. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent regulatory approval.

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

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

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

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

supportive data. In addition, in the U.S., an FDA advisory committee or regulators may disagree with our data analysis, interpretations and conclusions at any point in the approval process, which could negatively impact the approval of our NDA or result in a decision by the Company not to proceed with an NDA submission for a product candidate based on feedback from regulators.

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

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

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

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

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

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

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

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

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

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

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

In May 2018, a new privacy regime, the General Data Protection Regulation ("GDPR") will take effect and immediately be binding across all member states of the European Economic Area ("EEA"). The GDPR increases our obligations with respect to clinical trials conducted in the EEA by expanding the definition of personal data to include coded data, and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR increases the scrutiny that clinical trial sites located in the EEA should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the U.S. The GDPR imposes substantial fines for breaches of data protection requirements, which can be up to four percent of global revenue or 20 million Euros, whichever is greater, and it also confers a private right of action on data subjects for breaches of data protection requirements. Compliance with these directives will be a rigorous and time-intensive process that may increase our cost of doing business, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation and reputational harm in connection with our European activities.

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

We have relied upon, and plan to continue to rely upon, third parties to conduct some aspects of our early stage research and pre-clinical and clinical development with respect to certain of our product candidates, including our follow-on exon-skipping product candidates, PPMO and gene therapy-based product candidates. Our third-party collaborators may not commit sufficient resources or adequately develop our programs for these candidates. If our third-party collaborators fail to commit sufficient resources to any of our product candidates or to carry out their contractual duties or obligations, our programs related to any particular product candidate could be delayed,

terminated, or unsuccessful. Furthermore, if we fail to make required payments to these third-party collaborators, including up-front, milestone, reimbursement or royalty payments, or to observe other obligations in our agreements with them, these third parties may not be required to perform their obligations under our respective agreements with them and may have the right to terminate such agreements.

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

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

In addition, the use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that this information will be misappropriated. Furthermore, if these third parties cease to continue operations and we are not able to quickly find a replacement provider or we lose information or items associated with our product candidates, our development programs may be delayed. Although we carefully manage our relationships with our third-party collaborators and CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

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

We have historically relied on U.S. government contracts and awards to fund and support certain development programs. The July 2010 U.S. 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. In addition, the U.S. government may have the right to develop all or some parts of product candidates that we have developed under a U.S. government contract after such contract has terminated or expired.

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

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

- recruit and retain employees, consultants or contractors with the required level of expertise;
- recruit and retain sufficient patients needed to conduct a clinical trial;
- enroll and retain participants, which is a function of many factors, including the size of the relevant population, the proximity of participants to clinical sites, activities of patient advocacy groups, the eligibility criteria for the trial, the existence of competing clinical trials, the availability of alternative or new treatments, side effects from the therapy, lack of efficacy, personal issues and ease of participation;
- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the CROs involved in the clinical trial;
- negotiate contracts and other related documents with clinical trial parties and institutional review boards, such as informed consents, CRO agreements and site agreements, which can be subject to extensive negotiations that could cause significant delays in the clinical trial process, with terms possibly varying significantly among different trial sites and CROs and possibly subjecting the Company to various risks;

- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;
- manage or resolve unforeseen adverse side effects during a clinical trial;
- conduct the clinical trials in a cost-effective manner, including managing foreign currency risk in clinical trials conducted in foreign jurisdictions and cost increases due to unforeseen or unexpected complications such as enrollment delays, or needing to outsource certain Company functions during the clinical trial; and
- execute clinical trial designs and protocols approved by regulatory authorities without deficiencies.

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

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

We incurred an operating loss of $70.1 million and $204.3 million for the three and nine months ended September 30, 2018, respectively. Our accumulated deficit was $1.4 billion as of September 30, 2018. Although we launched EXONDYS 51 in the U.S. in September 2016, we believe that it will take us some time to attain profitability and positive cash flow from operations. We have generally incurred expenses related to research and development of our technologies and product candidates, from general and administrative expenses that we have incurred while building our business infrastructure. We anticipate that our expenses will increase substantially if and/or as we:

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

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

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

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

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

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

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

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

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

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

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

The Tax Cuts and Jobs Act (the “TCJA”) was enacted on December 22, 2017 in the United States. The TCJA contains significant changes to corporate taxation, including reduction of the U.S. corporate tax rate from 35% to 21%, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, limitation of the tax deduction for interest expense, immediate deductions for certain new investments instead of deductions for depreciation expense over time,

and modifying or repealing many business deductions and credits.

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

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

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

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

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

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

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

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

We may engage in future acquisitions or collaborations with other entities that complement or expand our business. We may not be able to complete such transactions, and such transactions, if executed, may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We actively evaluate various strategic transactions on an ongoing basis, including licensing or acquiring complementary products, technologies or businesses. We may face competition from other companies in pursuing acquisitions and similar transactions in the biotechnology industry. Our ability to complete transactions may also be limited by applicable antitrust and trade regulation laws and regulations in the U.S. and foreign jurisdictions in which we or the operations or assets we seek to acquire carry on business. To the extent that we are successful in undertaking acquisitions or collaborations with other entities, such transactions may entail numerous risks, including increased operating expenses and cash requirements, assimilation of operations and products and/or product candidates, retention of key employees, diversion of our management's attention, uncertainties in our ability to maintain key business relationships of the acquired entities. In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense.

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

We currently directly hold various issued patents and patent applications, or have exclusive license or option rights to issued patents and patent applications, in each case in the U.S. as well as other countries that protect our platform technology, product and product candidates, including EXONDYS 51, golodirsen, casimersen, SRP-5051 as well as our gene therapy-based product candidates (micro-dystrophin and Galgt2). We anticipate filing additional patent applications both in the U.S. and in other countries. The patent process, however, is subject to numerous risks and uncertainties, and we can provide no assurance that we will be successful in obtaining and defending patents or in avoiding infringement of the rights of others. Even when our patent claims are allowed, the claims may not issue, or in the event of issuance, may not be sufficient to protect the technology owned by, optioned, or licensed to us or our collaborators. Even if our patents and patent applications do provide our product, product candidates and platform technology with a basis for exclusivity, we and our collaborators may not be able to develop or commercialize such product and product candidates (whether PMO-based or gene therapy-based) or platform technology due to patent positions held by one or more third parties.

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

The patent positions of pharmaceutical, biotechnology and other life sciences companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. This uncertainty is heightened for our PMO-based product and product candidates and gene therapy-based product candidates for which there has been little patent litigation involving such technologies. No consistent policy regarding the breadth of claims allowed in biotechnology patents has emerged to date in the U.S. and tests used for determining the patentability of patent claims in all technologies are in flux. In addition, there is no assurance as to the degree and range of protections any of our patents, if issued, may afford us or whether patents will be issued. Patents which may be issued to us may be subjected to further governmental review that may ultimately result in the reduction of their

scope of protection, and pending patent applications may have their requested breadth of protection significantly limited before being issued, if issued at all. The pharmaceutical, biotechnology and other life sciences patent situation outside the U.S. can be even more uncertain.

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

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

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

The full impact of several recent U.S. Supreme Court decisions relating to patent law is not yet known. For example, on March 20, 2012, in Mayo Collaborative Services, DBA Mayo Medical Laboratories, et al. v. Prometheus Laboratories, Inc., the Court held that several claims drawn to measuring drug metabolite levels from patient samples and correlating them to drug doses were not patentable subject matter. The decision appears to impact diagnostics patents that merely apply a law of nature via a series of routine steps and it has created uncertainty around the ability to patent certain biomarker-related method claims. Additionally, on June 13, 2013, in Association for Molecular Pathology v. Myriad Genetics, Inc., the Court held that claims to isolated genomic DNA are not patentable, but claims to complementary DNA molecules were held to be valid. The effect of the decision on patents for other isolated

natural products is uncertain and, as with the Leahy-Smith Act, these decisions could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

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

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

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

- obtain rights or licenses from others, which might not be available on commercially reasonable terms or at all;
- abandon development of an infringing product candidate;

- redesign EXONDYS 51, product candidates or processes to avoid infringement;
- pay damages; and/or
- defend litigation or administrative proceedings which might be costly whether we win or lose, and which could result in a substantial diversion of financial and management resources.

Any of these events could substantially harm our potential earnings, financial condition and operations. The patent landscape of our product candidates (whether PMO-based or gene therapy-based) is continually evolving and multiple parties, including both commercial entities and academic institutions, may have rights to claims or may be pursuing additional claims that could provide these parties a basis to assert that EXONDYS 51 or our product candidates infringe on the intellectual property rights of such parties. Similarly, we may be able to assert that certain activities engaged in by these parties infringe on our current or future patent rights. To the extent that we enforce our patents, an alleged infringer may deny infringement and/or counter-claim that our patents are not valid, and if successful, could negatively impact our patent estate. 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 gene therapy technology and other technologies, or that are developing alternative approaches to or therapeutics for the disease indications on which we are focused. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with EXONDYS 51 or our product candidates. For example, we believe that companies including Alnylam Pharmaceuticals, Inc., Ionis Pharmaceuticals, Inc. (formerly Isis Pharmaceuticals, Inc.), Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Wave Life Sciences, Daiichi Sankyo and Nippon Shinyaku share a focus on RNA-targeted drug discovery and development. Competitors with respect to EXONDYS 51 or our product candidates (whether PMO-based or gene therapy-based) include Nippon Shinyaku, Daiichi Sankyo, Wave Life Sciences, Solid, Pfizer, Shire plc; and other companies such as PTC have also been working on DMD programs. Additionally, several companies and institutions have entered into collaborations or other agreements for the development of product candidates, including mRNA, gene therapy and gene editing (CRIPSR and AAV, among others) and small molecule therapies that are potential competitors for therapies being developed in the muscular dystrophy, neuromuscular and rare disease space, including, but not limited to, Biogen Inc., Ionis, Alexion Pharmaceuticals, Inc., Sanofi, Shire, Eli Lilly, Alnylam Pharmaceuticals, Inc., Moderna Therapeutics, Inc., Summit, Akashi, Catabasis, Capricor Therapeutics, Oxford University, Exonics Therapeutics, and Editas Medicine. Although BioMarin announced on May 31, 2016 its intent to discontinue clinical and regulatory development of drisapersen as well as its other clinical stage candidates, BMN 044, BMN 045 and BMN 053, then-currently in Phase 2 studies for distinct forms of DMD, it further announced its intent to continue to explore the development of next generation oligonucleotides for the treatment of DMD.

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

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

- develop safer or more effective products;

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

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

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

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

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

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

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

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

In the ordinary course of business we may, and in some cases have, become involved in lawsuits and other disputes such as securities claims, intellectual property challenges, including interferences declared by the USPTO, and

employee matters. It is possible that we may not prevail in claims made against us in such disputes even after expending significant amounts of money and company resources in defending our positions in such lawsuits and disputes. The outcome of such lawsuits and disputes is inherently uncertain and may have a negative impact on our business, financial condition and results of operations.

Risks Related to Our Common Stock

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

The market prices for and trading volumes of securities of biotechnology companies, including our securities, has historically been volatile. Our stock has had significant swings in trading prices, in particular in connection with our public communications regarding feedback received from regulatory authorities. For example, over the last thirty months, our stock has increased as much as 74% in a single day or decreased as much as 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 trials for potential product candidates;
- delays in entering or failing to enter into strategic relationships with respect to development and/or commercialization of EXONDYS 51 or our product candidates or entry into strategic relationships on terms that are not deemed to be favorable to our Company;
- technological innovations, product development or additional commercial product introductions by ourselves or competitors;
- changes in applicable government regulations or regulatory requirements in the approval process;
- developments concerning proprietary rights, including patents and patent litigation matters, such as developments in the interferences declared by the USPTO, including in the near term any outcomes of ongoing interference proceedings and over the longer term the outcomes from any related appeals;
- public concern relating to the commercial value, efficacy or safety of any of our products;
- our ability to obtain funds, through the issuance of equity or equity linked securities or incurrence of debt, or other corporate transactions;

- comments by securities analysts;
- developments in litigation such as the stockholder lawsuits against us;
- changes in senior management; or
- general market conditions in our industry or in the economy as a whole.

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

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

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

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

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

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

- prohibition on stockholder action by written consent;

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

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

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

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

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

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

- timing of purchase orders;
- changes in coverage and reimbursement policies of health plans and other health insurers, especially in relation to those products that are currently manufactured, under development or identified for future development by us;
- re-authorizations processes that may be required for patients who initially obtained coverage by third parties, including government payors, managed care organizations and private health insurers;
- transition from temporary billing codes established by the Centers for Medicare & Medicaid Services (CMS) to permanent medical codes;
- timing of approval of applications filed with the FDA;
- timing of product launches and market acceptance of products launched;
- changes in the amounts spent to research, develop, acquire, license or promote new and existing products;
- results of clinical trial programs;
- serious or unexpected health or safety concerns with our product or product candidates;
- introduction of new products by others that render our product obsolete or noncompetitive;
- the ability to maintain selling prices and gross margin on our product;
- increases in the cost of raw materials contained within our product;
- manufacturing and supply interruptions, including product rejections or recalls due to failure to comply with manufacturing specifications;
- timing of revenue recognition relating to our distribution agreements;

- the ability to protect our intellectual property from being acquired by other entities;
- the ability to avoid infringing the intellectual property of others; and
- the addition or loss of customers.

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

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

As of September 30, 2018, there were approximately 66.7 million shares of common stock outstanding and outstanding awards to purchase 9.2 million shares of common stock under various incentive stock plans. Additionally, as of September 30, 2018, there were approximately 4.4 million shares of common stock available for future issuance under our 2018 Equity Incentive Plan, approximately 0.2 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan, and approximately 1.0 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan. We may issue additional common stock and warrants from time to time to finance our operations. We may also issue additional shares to fund potential acquisitions or in connection with additional stock options or other equity awards granted to our employees, officers, directors and consultants under our 2018 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. The issuance of additional shares of common stock or warrants to purchase common stock and the perception that such issuances may occur or exercise of outstanding warrants or stock options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

Risks Related to Our Convertible Senior Notes

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

In 2017, we issued $570 million aggregate principal amount of Notes. Our ability to make scheduled payments of the principal of, to pay interest on, or to refinance our indebtedness, including the Notes, depends on our future performance, which is subject to many factors, including, economic, financial, competitive and other, beyond our control. We do not expect our business to be able to generate cash flow from operations, in the foreseeable future, sufficient to service our debt and make necessary capital expenditures and may therefore be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be onerous or highly dilutive. Our ability to refinance our Notes, which are non-callable and mature in 2024, will depend on the capital markets and our financial condition at such time. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations, and limit our flexibility in planning for and reacting to changes in our business.

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

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

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

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

In connection with establishing their initial hedges of the capped call transactions, these financial institutions or their respective affiliates entered into various derivative transactions with respect to our common stock and/or to purchase our common stock. The financial institutions, or their respective affiliates, may modify their hedge positions by entering into or unwinding various derivatives with respect to our common stock and/or purchasing or selling our common stock or other securities of ours in secondary market transactions prior to the maturity of the Notes. This activity could also cause or avoid an increase or a decrease in the market price of our common stock or the Notes, which could affect the value of our common stock.

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

None.

Item 6. Exhibits.

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

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated Form	File No.	Exhibit	Filing Date	Provided Herewith
10.1†	Form of Stock Option Award Agreement under Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan					X
10.2†	Form of Restricted Stock Award Agreement under Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan					X
10.3†	Form of Restricted Stock Unit Award Agreement under Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan					X
10.4†	Form of Stock Appreciation Right Award Agreement under Sarepta Therapeutics, Inc. 2018 Equity Incentive Plan					X
31.1	Certification of the Company's Chief Executive Officer, Douglas S. Ingram, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company's Executive Vice President, Chief Financial Officer and Chief Business Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1*	Certification of the Company's Chief Executive Officer, Douglas S. Ingram, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2*	Certification of the Company's Executive Vice President, Chief Financial Officer and Chief Business Officer, Sandesh Mahatme, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	XBRL Instance Document.					X
101.SCH	XBRL Taxonomy Extension Schema Document.					X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.					X

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

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

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

SAREPTA THERAPEUTICS, INC.

(Registrant)

Date: October 31, 2018 By: /s/ DOUGLAS S. INGRAM
Douglas S. Ingram
President and Chief Executive Officer
(Principal Executive Officer)

Date: October 31, 2018 By: /s/ SANDESH MAHATME
Sandesh Mahatme
Executive Vice President,
Chief Financial Officer and
Chief Business Officer
(Principal Financial and Accounting Officer)