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, 2019

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

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol	Name of exchange on which registered
Common Stock, $0.0001 par value per share	SRPT	The NASDAQ Global Select Market

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:

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	74,543,123
(Class)	**(Outstanding as of November 4, 2019)**

SAREPTA THERAPEUTICS, INC.
FORM 10-Q
INDEX

Item 1. Financial Statements

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

	As of September 30, 2019	As of December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 724,829	$ 370,829
Short-term investments	324,063	803,083
Accounts receivable	68,032	49,044
Inventory	166,360	125,445
Other current assets	79,015	77,782
Total current assets	1,362,299	1,426,183
Property and equipment, net of accumulated depreciation of $44,701 and $28,149 as of September 30, 2019, and December 31, 2018, respectively	119,532	97,024
Intangible assets, net of accumulated amortization of $5,091 and $3,852 as of September 30, 2019, and December 31, 2018, respectively	11,975	11,574
Right of use asset, net	39,493	—
Other assets	169,171	107,294
Total assets	$ 1,702,470	$ 1,642,075
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 81,946	$ 33,829
Accrued expenses	121,663	134,095
Deferred revenue	3,303	3,303
Other current liabilities	8,944	2,463
Total current liabilities	215,856	173,690
Long-term debt	436,421	420,554
Lease liabilities	49,741	—
Deferred rent and other	5,248	15,555
Total liabilities	707,266	609,799
Commitments and contingencies (Note 14)		
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; 74,504,835 and 71,071,887 issued and outstanding at September 30, 2019, and December 31, 2018, respectively	7	7
Additional paid-in capital	3,053,420	2,611,294
Accumulated other comprehensive income (loss)	75	(99)
Accumulated deficit	(2,058,298)	(1,578,926)
Total stockholders' equity	995,204	1,032,276
Total liabilities and stockholders' equity	$ 1,702,470	$ 1,642,075

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited, in thousands, except per share amounts)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2019	2018	2019	2018
Revenues:				
Product, net	$ 99,041	$ 78,486	$ 280,720	$ 216,619
Total revenues	99,041	78,486	280,720	216,619
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	13,037	8,741	41,019	21,058
Research and development	133,949	86,584	337,768	255,636
Selling, general and administrative	75,429	53,044	203,388	143,541
Acquired in-process research and development	—	—	173,240	—
Amortization of in-licensed rights	216	216	649	649
Total cost and expenses	222,631	148,585	756,064	420,884
Operating loss	(123,590)	(70,099)	(475,344)	(204,265)
Other loss:				
Other expense, net	(2,510)	(6,968)	(3,544)	(16,671)
Other loss	(2,510)	(6,968)	(3,544)	(16,671)
Loss before income tax expense	(126,100)	(77,067)	(478,888)	(220,936)
Income tax expense (benefit)	226	(674)	484	87
Net loss	(126,326)	(76,393)	(479,372)	(221,023)
Other comprehensive income (loss):				
Unrealized (losses) gains on investments	(7)	369	174	387
Total other comprehensive income (loss)	(7)	369	174	387
Comprehensive loss	$ (126,333)	$ (76,024)	$ (479,198)	$ (220,636)
Net loss per share - basic and diluted	$ (1.70)	$ (1.15)	$ (6.54)	$ (3.38)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	74,177	66,209	73,298	65,454

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(unaudited, in thousands)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2018	71,072	$ 7	$ 2,611,294	$ (99)	$ (1,578,926)	$ 1,032,276
Exercise of options for common stock	382	—	9,973	—	—	9,973
Grant of restricted stock awards and vest of restricted stock units, net of cancellations	35	—	—	—	—	—
Shares withheld for taxes	(7)	—	(889)	—	—	(889)
Issuance of common stock for cash, net of offering costs	2,604	—	365,264	—	—	365,264
Issuance of common stock under employee stock purchase plan	48	—	2,326	—	—	2,326
Stock-based compensation	—	—	16,139	—	—	16,139
Unrealized gains from available-for-sale securities	—	—	—	118	—	118
Net loss	—	—	—	—	(76,643)	(76,643)
Balance at March 31, 2019	74,134	$ 7	$ 3,004,107	$ 19	$ (1,655,569)	$ 1,348,564
Exercise of options for common stock	176	—	7,092	—	—	7,092
Grant of restricted stock awards and vest of restricted stock units, net of cancellations	18	—	—	—	—	—
Issuance of common stock for cash, net of offering costs	—	—	89	—	—	89
Stock-based compensation	—	—	19,762	—	—	19,762
Unrealized gains from available-for-sale securities	—	—	—	63	—	63
Net loss	—	—	—	—	(276,403)	(276,403)
Balance at June 30, 2019	74,328	$ 7	$ 3,031,050	$ 82	$ (1,931,972)	$ 1,099,167
Exercise of options for common stock	166	—	2,428	—	—	2,428
Grant of restricted stock awards and vest of restricted stock units, net of cancellations	1	—	—	—	—	—
Shares withheld for taxes	(34)	—	(3,448)	—	—	(3,448)
Issuance of common stock under employee stock purchase plan	44	—	2,753	—	—	2,753
Stock-based compensation	—	—	20,637	—	—	20,637
Unrealized losses from available-for-sale securities	—	—	—	(7)	—	(7)
Net loss	—	—	—	—	(126,326)	(126,326)
Balance at September 30, 2019	74,505	$ 7	$ 3,053,420	$ 75	$ (2,058,298)	$ 995,204

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY - PRIOR FISCAL YEAR
(unaudited, in thousands)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2017	64,792	$ 6	$ 2,006,598	$ (379)	$ (1,217,008)	$ 789,217
Exercise of options for common stock	653	1	11,887	—	—	11,888
Grant of restricted stock awards and vest of restricted stock units, net of cancellations	14	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	34	—	756	—	—	756
Stock-based compensation	—	—	10,526	—	—	10,526
Unrealized losses from available-for-sale securities	—	—	—	(264)	—	(264)
Net loss	—	—	—	—	(35,363)	(35,363)
Balance at March 31, 2018	65,493	$ 7	$ 2,029,767	$ (643)	$ (1,252,371)	$ 776,760
Exercise of options for common stock	872	—	21,743	—	—	21,743
Grant of restricted stock awards and vest of restricted stock units, net of cancellations	26	—	—	—	—	—
Shares withheld for taxes	(45)	—	(5,750)	—	—	(5,750)
Issuance of common stock under employee stock purchase plan	—	—	—	—	—	—
Stock-based compensation	—	—	15,279	—	—	15,279
Unrealized gains from available-for-sale securities	—	—	—	282	—	282
Net loss	—	—	—	—	(109,267)	(109,267)
Balance at June 30, 2018	66,346	$ 7	$ 2,061,039	$ (361)	$ (1,361,638)	$ 699,047
Exercise of options for common stock	323	—	7,094	—	—	7,094
Grant of restricted stock awards and vest of restricted stock units, net of cancellations	10	—	—	—	—	—
Shares withheld for taxes	(27)	—	(3,303)	—	—	(3,303)
Issuance of common stock under employee stock purchase plan	41	—	1,550	—	—	1,550
Stock-based compensation	—	—	11,484	—	—	11,484
Unrealized gains from available-for-sale securities	—	—	—	369	—	369
Net loss	—	—	—	—	(76,393)	(76,393)
Balance at September 30, 2018	66,693	$ 7	$ 2,077,864	$ 8	$ (1,438,031)	$ 639,848

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

	For the Nine Months Ended September 30,	
	2019	**2018**
Cash flows from operating activities:		
Net loss	$ (479,372)	$ (221,023)
Adjustments to reconcile net loss to cash flows from operating activities:		
Acquired in-process research and development	173,240	—
Depreciation and amortization	22,341	8,718
Amortization of investment discount	(7,449)	(4,742)
Loss from debt extinguishment	—	2,322
Non-cash interest expense	15,887	15,206
Stock-based compensation	56,538	37,289
Other	892	94
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable	(18,988)	(19,133)
Net increase in inventory	(40,915)	(32,211)
Net increase in other assets	(94,710)	(84,344)
Net increase in accounts payable, accrued expenses, deferred revenue and other liabilities	79,868	31,584
Net cash used in operating activities	(292,668)	(266,240)
Cash flows from investing activities:		
Purchase of property and equipment	(42,374)	(40,954)
Purchase of intangible assets	(2,048)	(2,633)
Purchase of available-for-sale securities	(984,225)	(651,387)
Purchases of restricted investment	—	(353)
Maturity and sale of available-for-sale securities	1,470,848	562,575
Acquisition of Myonexus Therapeutics, Inc., net of cash acquired	(172,556)	—
Net cash provided by (used in) investing activities	269,645	(132,752)
Cash flows from financing activities:		
Proceeds from sale of common stock, net of offering costs	365,353	—
Taxes paid related to net share settlement of equity awards	(4,337)	—
Proceeds from exercise of stock options and purchase of stock under the Employee Stock Purchase Program	24,572	43,031
Repayment of June 2015 and July 2017 Term Loan	—	(30,000)
Proceeds from revolving line of credit	—	217,722
Repayment of revolving line of credit	—	(218,631)
Repayments on mortgage loans	—	(1,265)
Payment of debt extinguishment	—	(1,990)
Net cash provided by financing activities	385,588	8,867
Increase (decrease) in cash, cash equivalents and restricted cash	362,565	(390,125)
Cash, cash equivalents and restricted cash:		
Beginning of period	370,829	599,827
End of period	$ 733,394	$ 209,702
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 724,829	$ 209,702
Restricted cash in other assets	8,565	—
Total cash, cash equivalents and restricted cash	$ 733,394	$ 209,702
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 4,275	$ 6,861
Supplemental schedule of non-cash investing activities and financing activities:		
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	$ 210	$ 234
Property and equipment included in accrued expenses	$ 2,117	$ 2,515

See accompanying notes to unaudited condensed consolidated financial statements.

1. ORGANIZATION AND NATURE OF BUSINESS

Sarepta Therapeutics, Inc. (together with its wholly-owned subsidiaries, "Sarepta" or the "Company") is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics, gene therapy and other genetic medicine approaches for the treatment of rare 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 U.S. Food and Drug Administration (the "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. In December 2018, the Company completed the submission of its rolling new drug application ("NDA") to the FDA seeking accelerated approval for golodirsen, the Company's PMO chemistry and exon-skipping technology to skip exon 53 of the DMD gene. On August 19, 2019, the Company received a Complete Response Letter ("CRL") from the FDA regarding the NDA. The CRL generally cites two concerns: the risk of infections related to intravenous infusion ports and renal toxicity seen in pre-clinical models of golodirsen and observed following administration of other antisense oligonucleotides.

As of September 30, 2019, the Company had approximately $1,058.5 million of cash, cash equivalents and investments, consisting of $724.8 million of cash and cash equivalents, $324.1 million of short-term investments, and $9.6 million of restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments as of the date of the issuance of this report is sufficient to fund its current operational plan for at least the next twelve months, though it may pursue raising 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 rare diseases.

These unaudited condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements and related notes for the year ended December 31, 2018 which are contained in the Company's Annual Report on Form 10-K filed with the U.S. Securities and Exchange Commission on February 28, 2019. The results for the three and nine months ended September 30, 2019 are not necessarily indicative of the results to be expected for the full year.

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.

Concentration of Credit Risk

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

As of September 30, 2019, 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 60 to 91 days. Outside of the U.S., the payment terms range between 45 and 150 days. Three individual customers accounted for 43%, 40% and 11% of net product revenues for the three months ended September 30, 2019 and 42%, 41% and 13% of net product revenues for the nine months ended September 30, 2019. Three individual customers accounted for 41%, 39% and 17% of net product revenues for the three months ended September 30, 2018 and 43%, 36% and 18% of net product revenues for the nine months ended September 30, 2018. Three individual customers accounted for 42%, 34% and 15% of accounts receivable from product sales as of September 30, 2019 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, 2019, the Company believes that such customers are of high credit quality.

As of September 30, 2019 the Company's cash was concentrated at three 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, 2018.

Leases

Effective January 1, 2019, the Company adopted ASC Topic 842, Leases ("ASC 842"), using the required modified retrospective approach and utilizing the effective date as its date of initial application, for which prior periods are presented in accordance with the previous guidance in ASC Topic 840, Leases ("ASC 840").

As a result of adopting ASC 842, the Company recorded lease right-of-use ("ROU") assets of $42.5 million and lease liabilities of $60.1 million as of January 1, 2019, primarily related to real estate leases, based on the present value of future lease payments on the date of adoption. The difference between the ROU assets and lease liabilities was due to previously recorded net deferred rent liabilities that were reclassified into the ROU assets. There was no impact to retained earnings upon adoption of ASC 842. Amounts related to finance leases were immaterial as of adoption and September 30, 2019.

At the inception of an arrangement, the Company determines whether the arrangement is or contains a lease based on the unique facts and circumstances present in the arrangement. Leases with a term greater than 12 months are recognized on the balance sheet as ROU assets and short-term and long-term lease liabilities, as applicable. The Company has elected not to recognize on the balance sheet leases with terms of 12 months or less. The Company typically only includes an initial lease term in its assessment of a lease arrangement. Options to renew a lease are not included in the Company's assessment unless there is reasonable certainty that the Company will renew. The Company monitors its plans to renew its material leases on a quarterly basis. In addition, the Company's lease agreements generally do not contain any residual value guarantees or restrictive covenants.

Operating lease liabilities and their corresponding ROU assets are recorded based on the present value of future lease payments over the expected remaining lease term. Lease cost for operating leases is recognized on a straight-line basis over the lease term as an operating expense. Certain adjustments to the ROU asset may be required for items such as lease prepayments or incentives received. The interest rate implicit in lease contracts is typically not readily determinable. As a result, the Company utilizes its incremental borrowing rate, which reflects the fixed rate at which the Company could borrow on a collateralized basis the amount of the lease payments in the same currency, for a similar term, in a similar economic environment. In transition to ASC 842, the Company utilized the remaining lease term of its leases in determining the appropriate incremental borrowing rate.

In accordance with ASC 842, components of a lease should be bifurcated between lease components and non-lease components. The fixed and in-substance fixed contract consideration identified must then be allocated based on the respective relative fair values to the lease components and non-lease components. However, ASC 842 provides entities with a practical expedient that allows them to make an accounting policy election to not separate lease and non-lease components by class of underlying asset. In using this expedient, entities would account for each lease component and the related non-lease component together as a single component. For new and amended real estate leases beginning after January 1, 2019, the Company has elected to account for the lease and non-lease components together for existing classes of underlying assets and allocates the contract consideration to the lease component only.

There have not been any other material changes to the Company's accounting policies through September 30, 2019.

Recent Accounting Pronouncements

In August 2018, the Financial Accounting Standards Board (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 and loss 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, 2019, 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, 2019, 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 June 2016, the FASB issued ASU No. 2016-13, "*Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments*". This ASU requires that credit losses be reported using an expected losses model rather than the incurred losses model that is currently used, and establishes additional disclosures related to credit risks. For available-for-sale debt securities with unrealized losses, this standard now requires allowances to be recorded instead of reducing the amortized cost of the investment. ASU 2016-13 limits the amount of credit losses to be recognized for available-for-sale debt securities to the amount by which carrying value exceeds fair value and requires the reversal of previously recognized credit losses if fair value increases. ASU 2016-13 will be effective for fiscal years beginning after December 15, 2019 with early adoption permitted, and requires adoption using a modified retrospective approach, with certain exceptions. Based on the composition of the Company's investment portfolio as of September 30, 2019, current market conditions and historical credit loss activity, the adoption of this standard is not expected to have a material impact on the Company's consolidated financial statements. Additionally, for trade receivables, due to their short duration and the credit profile of the Company's customers, the effect of transitioning from the incurred losses model to the expected losses model is not expected to be material.

3. LICENSE AND COLLABORATION AGREEMENTS

Myonexus Therapeutics

On May 3, 2018, the Company entered into a Warrant to Purchase Common Stock Agreement ("Warrant Agreement") with Myonexus Therapeutics, Inc. ("Myonexus"), a clinical-stage gene therapy biotechnology company that was developing gene therapies for Limb-Girdle muscular dystrophies ("LGMD"). 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.

On February 27, 2019, the Company announced that it exercised the exclusive option to acquire Myonexus. The final exercise price as negotiated between the Company and Myonexus was $165.0 million. In addition, the Company incurred transaction fees and other fees associated with the exercise of approximately $8.8 million. The Company may also be required to make up to $200.0 million in additional payments to selling shareholders of Myonexus based on the achievement of certain sales- and regulatory-related milestones. The acquisition closed on April 4, 2019.

As a result of the acquisition, the Company added five LGMD gene therapy programs, including MYO-101, MYO-102 and MYO-201 that are currently in Phase 1/2 clinical trials, to its research and development portfolio. The acquisition of Myonexus has been accounted for as an asset acquisition as substantially all of the fair value of the gross assets acquired is concentrated in a group of similar identifiable assets (the five LGMD gene therapy programs).

Additionally, the Company assessed whether any of the contingent payments met the definition of a derivative under ASC 815 and, therefore, should be accounted for as contingent consideration. The Company identified that one regulatory-related milestone (not solely based on drug approval by the FDA) met the definition of a derivative. As a result, the Company recorded a contingent consideration liability of $4.5 million at the acquisition date. Any changes in the fair value of the contingent consideration liability after the acquisition date will be reported in the Company's statement of operations. This amount was estimated through a probability-weighted expected return method that incorporated industry-based probability adjusted assumptions relating to the achievement of the milestone and thus the likelihood of making the payments. This fair value measurement was based upon significant inputs not observable in the market and therefore represented a Level 3 fair value measurement. The Company did not assume any other liabilities as a result of the acquisition.

The following table summarizes the total consideration for the acquisition and the value of assets acquired and liability assumed:

Consideration		
Purchase price	$	165,000
Transactions costs and other fees		8,753
Contingent consideration		4,500
Total consideration	$	178,253
Assets Acquired		
Cash and cash equivalents	$	1,197
Prepaids		3,816
In-process research and development		173,240
Total assets acquired	$	178,253
Liability Assumed		
Contingent consideration		4,500
Total liability assumed	$	4,500

The acquired in-process research and development asset relates to the LGMD asset group. Due to the stage of development of this asset group, significant risk remains, and it is not yet probable that there is future economic benefit from this asset. Absent successful clinical results and regulatory approval, there is no alternative future use associated with the LGMD asset group. Accordingly, the value of this asset of $173.2 million was immediately expensed to research and development expense during the three months ended June 30, 2019.

The portion of the $200.0 million in contingent payments related to the sales milestone will be accrued when and if the sales milestone becomes probable of being achieved, and the related payment will be capitalized and amortized over the life of the patent. As of September 30, 2019, the sales milestone was not probable of being achieved and, therefore, no assets or expense were recognized.

Summit

On October 3, 2016, the Company entered into an exclusive Collaboration and License Agreement (the "Collaboration Agreement") with Summit (Oxford) Ltd. ("Summit"), which grants us the exclusive right to commercialize products in Summit's utrophin modulator pipeline in the EU, Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States. 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. On August 16, 2019, the Company terminated the Collaboration Agreement. As of September 30, 2019, the Company had $0.5 million remaining to be paid to Summit in accordance with the agreement. There were no additional charges associated with the termination of the agreement.

Milestone Obligations

The Company has license and collaboration 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, 2019, the Company may be obligated to make up to $1,508.7 million of future development, regulatory, commercial, and up-front royalty milestone payments associated with its license and collaboration agreements. For the three and nine months ended September 30, 2019, the Company recognized up-front and milestone expenses of $10.2 million and $25.7 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, 2018, the Company recognized up-front and milestone expenses 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.

4. FAIR VALUE MEASUREMENTS

The Company has certain financial assets and liabilities 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 and liabilities 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, 2019			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 119,538	$ 119,538	$ —	$ —
Commercial paper	9,984	—	9,984	—
Government and government agency bonds	725,820	725,820	—	—
Corporate bonds	9,998	9,998	—	—
Strategic equity investments	31,834	1,834	—	30,000
Certificates of deposit	1,001	1,001	—	—
Total assets	$ 898,175	$ 858,191	$ 9,984	$ 30,000
Liabilities				
Contingent consideration	$ 5,200	$ —	$ —	$ 5,200
Total liabilities	$ 5,200	$ —	$ —	$ 5,200

	Fair Value Measurement as of December 31, 2018			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 42,920	$ 42,920	$ —	$ —
Commercial paper	125,907	—	125,907	—
Government and government agency bonds	760,235	760,235	—	—
Corporate bonds	43,468	43,468	—	—
Strategic equity investments	31,739	1,739	—	30,000
Certificates of deposit	1,001	1,001	—	—
Total	$ 1,005,270	$ 849,363	$ 125,907	$ 30,000

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, the Company's strategic investment in Lysogene S.A. and certificates of deposit. Certain of the government and government agency bonds are traded fixed income securities and are presented as cash equivalents on the unaudited condensed consolidated balance sheets as of September 30, 2019.

The Company's assets with fair value categorized as Level 2 within the fair value hierarchy consist of commercial paper. 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 asset with fair value categorized as Level 3 within the fair value hierarchy consists of a strategic investment in Series A preferred stock of Lacerta Therapeutics, Inc. ("Lacerta") as more fully described in *Note 3, License and Collaboration Agreements* of the Company's Annual Report on Form 10-K for the year ended December 31, 2018. The fair value of the asset was initially based on a cost approach corroborated by the Black-Scholes option pricing model. At the end of each reporting period, the fair value will be adjusted if Lacerta issues similar or identical equity securities or when there is a triggering event for impairment. There were no changes in the fair value of the Lacerta strategic investment from December 31, 2018.

The Company's contingent consideration liability with fair value categorized as Level 3 within the fair value hierarchy relate to the regulatory-related contingent payments to Myonexus selling shareholders as well as to an academic institution under a separate license agreement that meet the definition on a derivative. For more information related to Myonexus, please read *Note 3, License and Collaboration Agreements.* This amount was estimated through an income approach based on the probability-weighted expected cash flows that incorporated industry-based probability adjusted assumptions relating to the achievement of the milestone and thus the likelihood of making the payments. This fair value measurement was based upon significant inputs not observable in the market and therefore represented a Level 3 measurement. At the end of each reporting period, the fair value is adjusted to reflect the most current assumptions through earnings. There were no changes in the fair value of the contingent consideration since the liability was initially recorded in the three months ended June 30, 2019. As of September 30, 2019, the contingent consideration was recorded as a non-current liability on the Company's unaudited condensed consolidated balance sheets.

The carrying amounts reported in the unaudited condensed consolidated balance sheets for cash and cash equivalents, accounts receivable and accounts payable approximated fair value because of the short-term maturity of these financial instruments.

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, 2019	As of December 31, 2018
	(in thousands)	
Money market funds	$ 119,538	$ 42,920
Government and government agency bonds	421,739	111,587
Commercial paper	—	14,940
Total	$ 541,277	$ 169,447

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, 2019 and December 31, 2018 was approximately one and two 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, 2019			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)			
Cash and money market funds	$ 303,090	$ —	$ —	$ 303,090
Commercial paper	9,984	—	—	9,984
Government and government agency bonds	725,749	74	(3)	725,820
Corporate bonds	9,994	4	—	9,998
Total cash, cash equivalents and short-term investments	$ 1,048,817	$ 78	$ (3)	$ 1,048,892
As reported:				
Cash and cash equivalents	$ 724,808	$ 24	$ (3)	$ 724,829
Short-term investments	324,009	54	—	324,063
Total cash, cash equivalents and short-term investments	$ 1,048,817	$ 78	$ (3)	$ 1,048,892

	As of December 31, 2018			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)			
Cash and money market funds	$ 244,302	$ —	$ —	$ 244,302
Commercial paper	125,907	—	—	125,907
Government and government agency bonds	760,258	12	(35)	760,235
Corporate bonds	43,544	—	(76)	43,468
Total cash, cash equivalents and short-term investments	$ 1,174,011	$ 12	$ (111)	$ 1,173,912
As reported:				
Cash and cash equivalents	$ 370,827	$ 3	$ (1)	$ 370,829
Short-term investments	803,184	9	(110)	803,083
Total cash, cash equivalents and short-term investments	$ 1,174,011	$ 12	$ (111)	$ 1,173,912

6. ACCOUNTS RECEIVABLE AND RESERVES FOR PRODUCT SALES

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

	As of September 30, 2019	As of December 31, 2018
	(in thousands)	
Product sales, net of discounts and allowances	$ 67,260	$ 48,252
Government contract receivables	772	792
Total accounts receivable, net	$ 68,032	$ 49,044

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 Accruals	Total
	(in thousands)				
Balance, as of December 31, 2018	$ 1,378	$ 24,276	$ 538	$ 2,318	$ 28,510
Provision	7,291	32,310	3,541	7,729	50,871
Payments/credits	(7,838)	(22,053)	(3,009)	(5,975)	(38,875)
Balance, as of September 30, 2019	$ 831	$ 34,533	$ 1,070	$ 4,072	$ 40,506

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

	As of September 30, 2019	As of December 31, 2018
	(in thousands)	
Reduction to accounts receivable	$ 5,437	$ 2,364
Component of accrued expenses	35,069	26,146
Total reserves	$ 40,506	$ 28,510

7. INVENTORY

The following table summarizes the components of the Company's inventory for the period indicated:

	As of September 30, 2019	As of December 31, 2018
	(in thousands)	
Raw materials	$ 74,077	$ 71,313
Work in progress	90,489	47,279
Finished goods	1,794	6,853
Total inventory	$ 166,360	$ 125,445

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, 2019	As of December 31, 2018
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 47,795	$ 39,036
Prepaid clinical and pre-clinical expenses	9,062	9,706
Prepaid research expenses	3,957	1,932
Prepaid insurance	3,699	1,006
Leasehold improvement receivable	3,059	13,474
Prepaid maintenance services	2,794	2,994
Prepaid income tax	2,132	2,130
Prepaid commercial expenses	1,408	1,573
Other	5,109	5,931
Total other current assets	$ 79,015	$ 77,782

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

	As of September 30, 2019	As of December 31, 2018
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 117,811	$ 62,821
Strategic investments	31,834	31,739
Restricted cash and investments	9,566	1,001
Prepaid clinical expenses	4,499	7,541
Alternative minimum tax credit	3,367	3,367
Other	2,094	825
Total other non-current assets	$ 169,171	$ 107,294

9. ACCRUED EXPENSES

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

	As of September 30, 2019	As of December 31, 2018
	(in thousands)	
Product revenue related reserves	$ 35,069	$ 26,146
Accrued employee compensation costs	28,053	24,692
Accrued clinical and pre-clinical costs	15,595	11,396
Accrued contract manufacturing costs	15,250	15,794
Accrued professional fees	11,938	11,319
Accrued royalties	6,350	8,254
Accrued interest expense	3,183	1,045
Accrued property and equipment	2,117	5,421
Accrued research costs	778	1,070
Accrued collaboration cost sharing	541	2,167
Accrued milestone expense	152	24,020
Other	2,637	2,771
Total accrued expenses	$ 121,663	$ 134,095

10. STOCK-BASED COMPENSATION

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

	For the Three Months Ended September 30,				For the Nine Months Ended September 30,			
	2019		2018		2019		2018	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options	152,248	$ 51.64	359,182	$ 68.31	1,297,747	$ 72.50	2,011,863	$ 43.17
Restricted stock units	78,073	$ 99.52	5,990	$ 138.04	483,703	$ 132.45	175,410	$ 76.27
Restricted stock awards	—	$ —	10,500	$ 142.71	—	$ —	27,590	$ 98.57

Stock-based Compensation Expense

For the three months ended September 30, 2019 and 2018, total stock-based compensation expense was $20.6 million and $11.5 million, respectively. For the nine months ended September 30, 2019 and 2018, total stock-based compensation expense was $56.5 million and $37.3 million, respectively. The following table summarizes stock-based compensation expense by function included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2019	2018	2019	2018
	(in thousands)			
Research and development	$ 6,972	$ 3,260	$ 18,982	$ 10,349
Selling, general and administrative	13,665	8,224	37,556	26,940
Total stock-based compensation expense	$ 20,637	$ 11,484	$ 56,538	$ 37,289

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 September 30,		For the Nine Months Ended September 30,	
	2019	2018	2019	2018
	(in thousands)			
Stock options	$ 13,941	$ 9,007	$ 39,175	$ 27,362
Restricted stock awards/units	5,233	1,987	14,333	8,654
Employee stock purchase plan	1,463	490	3,030	1,273
Total stock-based compensation expense	$ 20,637	$ 11,484	$ 56,538	$ 37,289

11. OTHER EXPENSE, NET

The following table summarizes other expense, net, for the periods indicated:

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2019	2018	2019	2018
	(in thousand)			
Interest expense	$ (7,518)	$ (10,681)	$ (22,279)	$ (26,460)
Interest income	1,293	1,477	5,758	5,236
Amortization of investment discount	4,089	2,295	13,002	5,123
Other expense	(374)	(59)	(25)	(570)
Total other expense, net	$ (2,510)	$ (6,968)	$ (3,544)	$ (16,671)

12. LEASES

The Company's operating leases for its Cambridge, Andover and Burlington, Massachusetts and Dublin and Columbus, Ohio facilities provide for scheduled annual rent increases throughout each lease's term. A summary of these leases are as follows:

In June 2013, the Company entered into a lease agreement (the "Cambridge Lease") for its headquarters located in Cambridge, Massachusetts. In April and September 2018 and June 2019, the Company entered into the seventh, eighth, and ninth amendments, respectively, to its Cambridge Lease which extended the original term of the lease to September 30, 2025 and increased the total rental space to approximately 170,929 square feet.

In March and July of 2018, the Company entered into sublease agreements in Andover, Massachusetts, to lease a total of 23,102 square feet of office and lab space. These subleases expire in December 2020.

In November 2018, the Company entered into a sublease agreement in Burlington, Massachusetts, to lease 44,740 square feet of office and lab space. The sublease expires in January 2022.

In December 2018, the Company entered into lease agreements in Dublin and Columbus, Ohio, to lease 22,600 square feet and 77,679 square feet, respectively, of office and lab space. The leases expire in November 2019 and June 2026, respectively.

The adoption of ASC 842 resulted in the recognition of operating lease liabilities and ROU assets of $60.1 million and $42.5 million, respectively, on the Company's balance sheet relating to its leases for its corporate headquarters and its office and lab space on the January 1, 2019 transition date. Further, the Company reclassified upon adoption $18.0 million of deferred rent which served to reduce the ROU assets recognized on the balance sheet, in accordance with the transition guidance.

As of September 30, 2019, operating lease assets were $39.5 million and operating lease liabilities were $57.3 million. Amounts related to financing leases were immaterial. The following table contains a summary of the lease costs recognized under Topic 842 and other information pertaining to the Company's operating leases for the three and nine months ended September 30, 2019:

Operating Lease (in thousands)	For the three months ended September 30, 2019	For the nine months ended September 30, 2019
Lease cost		
Operating lease cost	2,606	7,738
Variable lease cost	1,008	2,946
Total lease cost	$ 3,614	$ 10,684
Other information		
Operating lease payments		7,606
Operating lease liabilities arising from obtaining ROU assets		—
Weighted average remaining lease term		5.7
Weighted average discount rate		7.50%

Future minimum lease payments under the Company's non-cancelable operating leases as of September 30, 2019, are as follows:

Maturity of lease liability (in thousands)	As of September 30, 2019
2019 (October - December)	$ 2,809
2020	11,718
2021	12,890
2022	11,080
2023	11,230
Thereafter	21,126
Total minimum lease payments	70,853
Less: imputed interest	(13,516)
Total operating lease liabilities	$ 57,337
Included in the condensed consolidated balance sheet:	
Current portion of lease liabilities within other current liabilities	$ 7,596
Lease liabilities	49,741
Total operating lease liabilities	$ 57,337

Future minimum lease payments under the Company's non-cancelable operating leases as of December 31, 2018, are as follows:

Maturity of lease liability (in thousands)	As of December 31, 2018
2019	$ 11,588
2020	11,395
2021	12,558
2022	10,757
2023	10,898
Thereafter	20,524
Total minimum lease payments	$ 77,720

13. 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, 2019 and 2018, 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 September 30,		For the Nine Months Ended September 30,	
	2019	2018	2019	2018
	(in thousands, except per share amounts)			
Net loss	$ (126,326)	$ (76,393)	$ (479,372)	$ (221,023)
Weighted-average common shares outstanding - basic	74,177	66,209	73,298	65,454
Effect of dilutive securities*	—	—	—	—
Weighted-average common shares outstanding - diluted	74,177	66,209	73,298	65,454
Net loss per share - basic and diluted	$ (1.70)	$ (1.15)	$ (6.54)	$ (3.38)

* For the three and nine months ended September 30, 2019 and 2018, stock options, RSAs, RSUs, and ESPP to purchase 9.6 million and 9.2 million shares of the Company's common stock, respectively, were excluded from the diluted net loss per share calculation as their effect would have been anti-dilutive. The Company accounts for the effect of the 2024 Notes on diluted net earnings per share using the if-converted method as they may be settled in cash or shares at the Company's option. While the closing price on September 30, 2019 exceeded the conversion price of $73.42, the potential shares issuable under the 2024 Notes were excluded from the calculation of diluted loss per share as they were anti-dilutive using the if-converted method. In the period of conversion, the 2024 Notes will have no impact on diluted net earnings (loss) if they are settled in cash and will have an impact on diluted earnings per share if the Notes are settled in shares upon conversion and when the Company in an income position.

14. COMMITMENTS AND CONTINGENCIES

Manufacturing Obligations

Brammer Bio MA, LLC

The Company entered into a Development, Commercial Manufacturing and Supply Agreement (the "Brammer Manufacturing Agreement") and, subsequently, entered into the first amendment (the "Amendment") to the Brammer Manufacturing Agreement (collectively, "Brammer Supply Agreements") with Brammer Bio MA, LLC ("Brammer") in June 2018 and May 2019, respectively. Pursuant to the terms of the Brammer Supply Agreements, Brammer agreed to provide the Company with access to clinical and commercial manufacturing capacity for its gene therapy programs. For more information related to the Brammer Manufacturing Agreement, please read *Note 21, Commitments and Contingencies* of the Company's Annual Report on Form 10-K for the year ended December 31, 2018.

As a result of the Amendment: (i) the Company now has access to substantially all of the related facility's capacity, subject to certain minimum and maximum volume limitations, (ii) the Company was required to make a $6.0 million advance payment to Brammer upon execution of the Amendment, and (iii) the quarterly capacity access fee payments due to Brammer throughout the term of the agreement increased from $10.0 million to $13.3 million, starting January 1, 2020. However, through December 31, 2019, a reduced quarterly capacity access fee will be in effect as Brammer works towards achieving full capacity at its facility. In addition, the application of certain prepayments will reduce the quarterly capacity access fees paid through 2021.

Upon execution of the Amendment, the Company determined that the Brammer Supply Agreements contain an embedded lease because the Company now has the right to direct the use of the facility and related equipment therein. Further, the Company determined that it does not control the facility or related equipment during construction and, thus, the lease does not fall in the scope of "build-to-suit" accounting. The lease had not commenced as of September 30, 2019. Accordingly, total cumulative payments made to Brammer of $68.3 million have been recorded as other assets and will be considered in the initial measurement of the cost of the right-of-use asset at the lease commencement date. The Company expects the lease commencement to occur in the three months ended December 31, 2019, and upon commencement, the Company expects a material right-of-use asset and lease liability will be recorded.

Paragon Bioservices, Inc.

In October 2018, the Company entered into a manufacturing collaboration agreement (the "Collaboration Agreement") with Paragon Bioservices, Inc. ("Paragon"). Pursuant to the terms of the Collaboration Agreement, Paragon agreed to provide the Company with two dedicated clean room suites and an option to gain access to two additional clean room suites for its gene therapy programs. On February 22, 2019, the Company entered into a manufacturing and supply agreement (the "Paragon Supply Agreement") with Paragon. The Paragon Supply Agreement consists of two periods: the pre-launch period and the post-launch period. During the pre-launch period, the Company is obligated to purchase a minimum amount of $4.0 million of batches per quarter per clean room. During the post-launch period, on an annual basis, the Company is obligated to purchase a minimum number of batches per clean room. Both the Collaboration Agreement and the Supply Agreements will expire on December 31, 2024. On September 11, 2019, the Company exercised the option to gain access to additional clean room suites and is required to pay a reservation fee of $12.0 million during fiscal year 2020 in connection with the exercise of the option.

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

	As of September 30, 2019 (in thousands)
2019 (October - December)	$ 99,583
2020	264,955
2021	168,690
2022	57,204
2023	57,244
Thereafter	202,750
Total manufacturing commitments	$ 850,426

Additionally, should the Company obtain regulatory approval for any drug product candidate produced as a part of the Company's manufacturing obligations above, additional minimum batch requirements with the respective manufacturing parties would be required.

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, on August 30, 2019, Plaintiff Andrew Salinger filed a putative class action complaint against the Company and two of its current officers, Douglas S. Ingram and Sandesh Mahatme (collectively, the "Defendants"), in the United States District Court for the Southern District of New York. The complaint alleges that the Defendants violated Section 10(b) of the Securities Exchange Act of 1934, as amended ("Exchange Act"), and SEC Rule 10b-5 promulgated thereunder, as well as Section 20(a) of the Exchange Act, in connection with the Company's disclosures related to golodirsen. The proposed class consists of all persons or entities who acquired Company securities between September 6, 2017 and August 19, 2019. We are unable to provide an estimate of possible loss or range of possible loss.

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, 2018 under the caption "Part II-Item 7 — Management's Discussion and Analysis of Financial Condition and Results of Operations". This discussion contains certain forward-looking statements, which are often identified by words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may," "estimate," "could," "continue," "ongoing," "predict," "potential," "likely," "seek" and other similar expressions, as well as variations or negatives of these words. These statements 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 new pharmaceutical products;*
- *our technologies and programs, including those with strategic partners, and their respective potential benefits, including the goal of exon skipping to create the production of an internally truncated but functional dystrophin protein and the goal of MYO-101 to restore the dystrophin associated protein complex;*
- *our belief that our partnerships with manufacturers will provide us access to additional commercial manufacturing capacity for our micro-dystrophin Duchenne muscular dystrophy ("DMD") gene therapy program, as well as a manufacturing platform for future gene therapy programs, and our belief that our current network of manufacturing partners are able to fulfil the requirements of our commercial plan;*
- *our plan to continue building out our network for commercial distribution in jurisdictions in which eteplirsen is approved;*
- *estimated timelines and milestones for 2019 and beyond, including our plan to evaluate the submission of a New Drug Application ("NDA") to the U.S. Food and Drug Administration ("FDA") for casimersen once we have further clarity on the Complete Response Letter we received from the FDA regarding the NDA for golodirsen and initiating a confirmatory trial for our micro-dystrophin gene therapy program using commercial supply of SRP-9001 in the first half of 2020;*
- *our plan to expand our pipeline in 2019 through internal research and development and through strategic transactions;*
- *our belief that the delivery of NT-3 may have applicability to other subtypes of Charcot-Marie-Tooth ("CMT") in addition to other muscle-wasting diseases;*
- *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 plan to evaluate future engagement with the European Medicines Agency (the "EMA") on potential next steps for EMA approval of eteplirsen;*
- *our ability to further secure long-term supply of EXONDYS 51 and our product candidates to satisfy our planned commercial, early access programs and clinical needs;*
- *the impact of regulations and regulatory decisions by the FDA and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations;*
- *the possible impact of any competing products on the commercial success of EXONDYS 51 and our product candidates and our ability to compete against such products;*
- *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 estimates regarding how long our currently available cash and cash equivalents will be sufficient to finance our operations and business plans and statements about our future capital needs;*
- *our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;*

- *our expectations relating to potential funding from government and other sources for the development of some of our product candidates;*
- *our expectation regarding the impact of environmental laws and regulations on our business; 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 therapeutic modalities for the treatment of rare diseases. Applying our proprietary, highly-differentiated and innovative technologies, and through collaborations with our strategic partners, we are developing potential therapeutic candidates for a broad range of diseases and disorders, including DMD, Limb-Girdle muscular dystrophies ("LGMDs"), Mucopolysaccharidosis type IIIA ("MPS IIIA") and Pompe.

Our first commercial product in the U.S., EXONDYS 51® (eteplirsen) Injection ("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. EXONDYS 51 uses our phosphorodiamidate morpholino oligomer ("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 of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to create the production of an internally truncated but functional dystrophin protein.

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

A summary description of our main product candidates, including those in collaboration with our strategic partners, is as follows:

- *Golodirsen* (SRP-4053) uses our PMO chemistry and exon-skipping technology to skip exon 53 of the DMD gene. Golodirsen is designed to bind to exon 53 of dystrophin pre-mRNA, resulting in exclusion, or "skipping", of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. 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 has also been evaluated in a Phase 1/2 trial, which has been completed (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 4053-101 interim trial results demonstrated statistical significance on all primary and secondary biological endpoints. In December 2018, we completed the submission of our rolling NDA to the FDA seeking accelerated approval for golodirsen. On August 19, 2019, we received a Complete Response Letter ("CRL") from the FDA regarding the NDA. The CRL generally cites two concerns: the risk of infections related to intravenous infusion ports and renal toxicity seen in pre-clinical models of golodirsen and observed following administration of other antisense oligonucleotides.
- *Casimersen* (SRP-4045) uses our PMO chemistry and exon-skipping technology to skip exon 45 of the DMD gene. Casimersen is designed to bind to exon 45 of dystrophin pre-mRNA, resulting in exclusion, or "skipping", of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 45 skipping. We are enrolling and dosing patients in ESSENCE, further described above. We have completed a dose titration portion (Phase 1) and the open-label portion (Phase 2) of a Sarepta sponsored Phase 1/2 clinical trial studying casimersen (Study 4045-101). On March 28, 2019, we announced results from our interim analysis of muscle biopsy endpoints comparing casimersen treatment to placebo in the ESSENCE study. We plan to evaluate the submission of an NDA for casimersen once we have further clarity on the CRL.

- *SRP-5051* uses our next-generation chemistry platform, peptide conjugated PMO ("PPMO"), and our exon-skipping technology to skip exon 51 of the DMD gene. SRP-5051, a PPMO, is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to create the production of an internally truncated but functional dystrophin protein. In the fourth quarter of 2017, we commenced a first-in-human, single ascending dose, study for the treatment of DMD in patients who are amenable to exon 51 skipping. We have also recently commenced an in-human, multiple ascending dose, study for the treatment of DMD in patients who are amenable to exon 51 skipping.
- *SRP-9001* (micro-dystrophin gene therapy program), in collaboration with Nationwide Children's Hospital ("Nationwide"), aims to express micro-dystrophin – a smaller but still functional version of dystrophin ("micro-dystrophin"). A unique, engineered micro-dystrophin is used because naturally-occurring dystrophin is too large to fit in an adeno-associated virus ("AAV") vector. In the fourth quarter of 2017, an investigational new drug ("IND") application for the micro-dystrophin gene therapy program was cleared by the FDA, and a Phase 1/2a clinical trial in individuals with DMD was initiated. On October 3, 2018, Nationwide presented positive results from the Phase 1/2a clinical trial in four individuals with DMD enrolled in the trial. On March 25, 2019, we presented 9-month functional and creatine kinase ("CK") data from baseline from these four individuals, and 12-months CK data from baseline from one of these individuals.

  In the fourth quarter of 2018, we commenced a placebo-controlled trial with the goal to establish the functional benefits of micro-dystrophin expressions. We plan to initiate a confirmatory trial using commercial supply of SRP-9001 in the first half of 2020, pending regulatory feedback.
- *MYO-101*. Myonexus Therapeutics, Inc. ("Myonexus"), our wholly-owned subsidiary, develops gene therapy programs for various forms of LGMDs. The most advanced of Myonexus' product candidates, MYO-101, is designed to treat LGMD type 2E by transferring 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. Myonexus commenced a Phase 1/2a trial of MYO-101 in the fourth quarter of 2018. On February 27, 2019, we announced positive two-month data from the first three-patient cohort dosed in the MYO-101 trial and on October 4, 2019, we announced positive nine-months data from these three patients.
- *GALGT2*. An additional gene therapy program for DMD and other muscular dystrophies, also in collaboration with Nationwide, aims to express the enzyme GALGT2 from an AAV vector. In the fourth quarter of 2017, the IND application for GALGT2 was cleared by the FDA, and a Phase 1/2a clinical trial testing GALGT2 for the treatment of DMD was initiated.
- *LYS-SAF 302*. We are collaborating with Lysogene S.A. ("Lysogene") to develop a gene therapy, LYS-SAF302, to treat MPS IIIA. The first patient has been dosed in AAVance, a global Phase 2/3 clinical trial of LYS-SAF302, aiming at evaluating the effectiveness of a one-time delivery of a AAVrh10 virus carrying the N-SGSH gene.
- *Neutrophin 3* (CMT Type 1A). A gene therapy program in collaboration with Nationwide that aims to express NT-3 encoding the NTF3 gene to treat CMT neuropathies, including CMT type 1A. We believe that the delivery of NT-3 gene may have applicability to other sub-types of CMT in addition to other muscle-wasting diseases.

Our pipeline includes programs in various stages of pre-clinical and clinical development, reflecting our aspiration to apply our multifaceted approach and expertise in precision genetic medicine to make a profound difference in the lives of patients suffering from rare diseases.

Manufacturing, Supply and Distribution

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. In 2017, we opened a facility in Andover, Massachusetts, which significantly enhanced 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. For our current and future manufacturing and supply needs, we have entered into certain manufacturing and supply arrangements with specialized contract manufacturing organizations (each a "CMO") to produce custom raw materials, the active pharmaceutical Ingredients ("APIs"), drug product and finished goods for our product candidates. All of our CMO partners have extensive technical expertise, GMP experience and experience manufacturing our specific technology.

For our commercial DMD program, we have commenced work with our existing manufacturers to increase product capacity from mid-scale to large-scale. While there are a limited number of companies that can produce raw materials and APIs in the quantities and with the quality and purity that we require for EXONDYS 51, based on our diligence to date, we believe our current network of manufacturing partners are able to fulfill these requirements, and are capable of expanding capacity as needed. Additionally, we have, and will continue to evaluate further relationships with additional suppliers to increase overall capacity as well as further reduce risks associated with reliance on a limited number of suppliers for manufacturing.

EXONDYS 51 is distributed in the U.S. through a limited network of home infusion specialty pharmacy providers that deliver the medication to patients and a specialty distributor that distributes EXONDYS 51 to hospitals and hospital outpatient clinics. 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 through our ex-U.S. early access programs ("EAP"). We plan to continue building out our network for commercial distribution in jurisdictions in which eteplirsen is approved.

Our gene therapy manufacturing capabilities have been greatly enhanced through partnerships with Brammer Bio LLC, which has recently been acquired by Thermo Fisher Scientific Inc. ("Brammer"), Paragon Bioservices, Inc., which has recently been acquired by Catalent, Inc. ("Paragon") and Aldevron LLC ("Aldevron"). We have adopted a hybrid development and manufacturing strategy in which we are building internal manufacturing expertise relative to all aspects of AAV-based manufacturing, including gene therapy and gene editing supply, while closely partnering with best-in-class manufacturing partners to expedite development and commercialization of our gene therapy programs. The partnership with Brammer will support our clinical and commercial manufacturing capacity for our micro-dystrophin DMD gene therapy programs and LGMD programs, while also acting as a manufacturing platform for potential future gene therapy programs. The collaboration integrates process development, clinical production and testing, and commercial manufacturing. Our partnership with Paragon will provide us access to additional commercial manufacturing capacity for our micro-dystrophin DMD gene therapy program, as well as a manufacturing platform for future gene therapy programs, such as LGMD. Aldevron will provide GMP-grade plasmid for our micro-dystrophin DMD gene therapy program and LGMD programs, as well as plasmid source material for future gene therapy programs, such as CMT, MPS IIIA, Pompe and other central nervous system diseases.

Manufacturers and suppliers of product candidates are subject to the FDA's current GMP ("cGMP") requirements and other rules and regulations prescribed by foreign regulatory authorities. We depend on our third-party partners for continued compliance with cGMP requirements and applicable foreign standards.

Cash, Cash Equivalents and Investments

As of September 30, 2019, we had approximately $1,058.5 million of cash, cash equivalents and investments, consisting of $724.8 million of cash and cash equivalents, $324.1 of short-term investments, and $9.6 million of long-term restricted cash and investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve months.

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

Critical Accounting Policies and Estimates

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

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

There have been no changes to our critical accounting policies and significant estimates as detailed in our Annual Report on Form 10-K for the year ended December 31, 2018.

Results of Operations for the Three and Nine Months Ended September 30, 2019 and 2018

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

	For the Three Months Ended September 30,			
	2019	2018	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$ 99,041	$ 78,486	$ 20,555	26%
Total revenues	99,041	78,486	20,555	26%
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	13,037	8,741	4,296	49%
Research and development	133,949	86,584	47,365	55%
Selling, general and administrative	75,429	53,044	22,385	42%
Amortization of in-licensed rights	216	216	—	(—)%
Total cost and expenses	222,631	148,585	74,046	50%
Operating loss	(123,590)	(70,099)	(53,491)	76%
Other loss:				
Other expense, net	(2,510)	(6,968)	4,458	(64)%
Loss before income tax expense (benefit)	(126,100)	(77,067)	(49,033)	64%
Income tax expense (benefit)	226	(674)	900	(134)%
Net loss	$ (126,326)	$ (76,393)	$ (49,933)	65%
Net loss per share - basic and diluted	$ (1.70)	$ (1.15)	$ (0.55)	48%

	For the Nine Months Ended September 30,			
	2019	2018	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$ 280,720	$ 216,619	$ 64,101	30%
Total revenues	280,720	216,619	64,101	30%
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	41,019	21,058	19,961	95%
Research and development	337,768	255,636	82,132	32%
Selling, general and administrative	203,388	143,541	59,847	42%
Acquired in-process research and development	173,240	—	173,240	NM*
Amortization of in-licensed rights	649	649	—	(—)%
Total cost and expenses	756,064	420,884	335,180	80%
Operating loss	(475,344)	(204,265)	(271,079)	133%
Other loss:				
Other expense, net	(3,544)	(16,671)	13,127	(79)%
Loss before income tax expense	(478,888)	(220,936)	(257,952)	117%
Income tax expense	484	87	397	NM*
Net loss	$ (479,372)	$ (221,023)	$ (258,349)	117%
Net loss per share - basic and diluted	$ (6.54)	$ (3.38)	$ (3.16)	94%

* NM = Not Meaningful

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). Our estimates take into consideration current contractual and statutory requirements. The amount of variable consideration 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 net loss in the period such variances become known. Net product revenues for EXONDYS 51 for the three and nine months ended September 30, 2019 increased by $20.6 million and $64.1 million compared with the three and nine months ended September 30, 2018, respectively. These increases 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, inventory costs that relate to sales of EXONDYS 51 and the related overhead costs. In addition to royalty payments to BioMarin Pharmaceuticals, Inc. (“BioMarin”), during the second quarter of 2019, we began to pay the University of Western Australia (“UWA”) a low-single-digit percentage royalty on net sales of products covered by issued patents licensed from UWA during the term of the Amended and Restated UWA License Agreement. Prior to receiving regulatory approval for EXONDYS 51 from the FDA in September 2016, we expensed manufacturing and material costs as research and development expenses. For EXONDYS 51 sold in the three and nine months ended September 30, 2019 and 2018, 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 expenses for the EXONDYS 51 sold would have been approximately $8.8 million and $9.5 million for the nine months ended September 30, 2019 and 2018, respectively.

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

	For the Three Months Ended September 30,			
	2019	2018	Change	Change
	(in thousands)		$	%
Inventory costs related to EXONDYS 51 sold and other costs	$ 6,687	$ 4,740	$ 1,947	41%
Royalty payments	6,350	4,001	2,349	59%
Total cost of sales	$ 13,037	$ 8,741	$ 4,296	49%

The cost of sales for the three months ended September 30, 2019 increased by $4.3 million, or 49%, compared with the same period in 2018. The increase was primarily driven by the following:

- $1.9 million and $2.3 million increases in inventory costs related to EXONDYS 51 sold and other costs and royalty payments to BioMarin and UWA, respectively, reflect increasing demand for EXONDYS 51.

	For the Nine Months Ended September 30,			
	2019	2018	Change	Change
	(in thousands)		$	%
Inventory costs related to EXONDYS 51 sold and other costs	$ 24,396	$ 10,245	$ 14,151	138%
Royalty payments	16,623	10,813	5,810	54%
Total cost of sales	$ 41,019	$ 21,058	$ 19,961	95%

The cost of sales for the nine months ended September 30, 2019 increased by $20.0 million, or 95%, compared with the same period in 2018. The increase was primarily driven by the following:

- $14.2 million and $5.8 million increases in inventory costs related to EXONDYS 51 sold and other costs and royalty payments to BioMarin and UWA, respectively, reflect increasing demand for EXONDYS 51; and,

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 that 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- and technology-related costs.

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

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

	For the Three Months Ended September 30,			
	2019	2018	Change $	Change %
	(in thousands)			
Gene therapies	$ 36,110	$ —	$ 36,110	NM*
Up-front, milestone, and other expenses	12,146	18,000	(5,854)	(33)%
Eteplirsen (exon 51)	12,703	8,738	3,965	45%
Casimersen (exon 45)	8,343	6,004	2,339	39%
Golodirsen (exon 53)	5,956	6,193	(237)	(4)%
PPMO platform	4,575	7,665	(3,090)	(40)%
Other projects	323	2,548	(2,225)	(87)%
Collaboration cost-sharing	—	1,570	(1,570)	(100)%
Internal research and development expenses	53,793	35,866	17,927	50%
Total research and development expenses	$ 133,949	$ 86,584	$ 47,365	55%

	For the Nine Months Ended September 30,			
	2019	2018	Change $	Change %
	(in thousands)			
Gene therapies	$ 76,294	$ —	$ 76,294	NM*
Eteplirsen (exon 51)	30,392	24,961	5,431	22%
Up-front, milestone, and other expenses	28,346	78,000	(49,654)	(64)%
Casimersen (exon 45)	21,237	21,342	(105)	(0)%
Golodirsen (exon 53)	17,012	20,598	(3,586)	(17)%
PPMO platform	14,018	16,461	(2,443)	(15)%
Other projects	3,123	3,952	(829)	(21)%
Collaboration cost-sharing	416	7,632	(7,216)	(95)%
Internal research and development expenses	146,930	82,690	64,240	78%
Total research and development expenses	$ 337,768	$ 255,636	$ 82,132	32%

*NM = Not Meaningful

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

	For the Three Months Ended September 30,			
	2019	2018	Change	Change
	(in thousands)		$	%
Clinical and manufacturing expenses	$ 65,002	$ 29,154	$ 35,848	123%
Compensation and other personnel expenses	21,808	14,061	7,747	55%
Up-front, milestone, and other expenses	12,146	18,000	(5,854)	(33)%
Facility- and technology-related expenses	12,104	4,852	7,252	149%
Stock-based compensation	6,972	3,260	3,712	114%
Professional services	6,656	3,627	3,029	84%
Pre-clinical expenses	1,812	7,141	(5,329)	(75)%
Collaboration cost-sharing	—	1,570	(1,570)	(100)%
Research and other	7,449	4,919	2,530	51%
Total research and development expenses	$ 133,949	$ 86,584	$ 47,365	55%

Research and development expenses for the three months ended September 30, 2019 increased by $47.4 million, or 55%, compared with the three months ended September 30, 2018. The increase was primarily driven by the following:

- $35.8 million increase in clinical and manufacturing expenses primarily due to a continuing ramp-up of our micro-dystrophin program, our ESSENCE program and initiation of certain post-market studies for EXONDYS 51. The increases were offset by a ramp-down of the PROMOVI trial in EXONDYS 51 and the Phase 1/2 trial in golodirsen;
- $7.7 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $5.9 million decrease in up-front, milestone, and other expenses primarily due to $10.0 million of an up-front payment due as a result of execution of a certain license agreement during the third quarter of 2019, as compared with a $10.0 million milestone payment to Myonexus as a result of the achievement of one of the development milestones in September 2018 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 a license agreement with Lacerta Therapeutics, Inc. (“Lacerta”) in August 2018;
- $7.3 million increase in facility- and technology-related expenses due to our continuing global expansion efforts as well as a change in methodology in allocation of technology expense;
- $3.7 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;
- $3.0 million increase in professional services primarily due to continuing accelerated company growth as a result of expansion of our research and development pipeline;
- $5.3 million decrease in pre-clinical expenses primarily due to completion of certain toxicology studies in our PPMO platform;
- $1.6 million decrease in collaboration cost sharing with Summit (Oxford) Ltd. as it is winding down activities on its Utrophin platform; and
- $2.5 million increase in research and other primarily driven by an increase in lab supplies as a result of an increase in headcount as well as sponsored research with academic institutions.

	For the Nine Months Ended September 30,			
	2019	2018	Change	Change
	(in thousands)		$	%
Clinical and manufacturing expenses	$ 146,773	$ 75,630	$ 71,143	94%
Compensation and other personnel expenses	63,527	34,471	29,056	84%
Facility- and technology-related expenses	34,216	11,292	22,924	203%
Up-front, milestone, and other expenses	28,346	78,000	(49,654)	(64)%
Stock-based compensation	18,982	10,349	8,633	83%
Professional services	16,292	12,108	4,184	35%
Pre-clinical expenses	9,826	15,223	(5,397)	(35)%
Collaboration cost-sharing	416	7,632	(7,216)	(95)%
Research and other	19,390	10,931	8,459	77%
Total research and development expenses	$ 337,768	$ 255,636	$ 82,132	32%

Research and development expenses for the nine months ended September 30, 2019 increased by $82.1 million, or 32%, compared with the nine months ended September 30, 2018. The increase was primarily driven by the following:

- $71.1 million increase in clinical and manufacturing expenses primarily due to a continuing ramp-up of our micro-dystrophin program, our ESSENCE program and initiation of certain post-market studies for EXONDYS 51. The increases were offset by a ramp-down of the PROMOVI trial in EXONDYS 51 and the Phase 1/2 trial in golodirsen;
- $29.1 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $22.9 million increase in facility- and technology-related expenses due to our continuing expansion efforts as well as a change in methodology in allocation of technology expense;
- $49.7 million decrease in up-front and milestone expenses primarily due to $25.5 million of up-front payments as a result of license agreements executed during the nine months ended September 30, 2019, as compared with an up-front payment of $60.0 million to Myonexus upon execution of the warrant to purchase common stock 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 a license agreement with Lacerta in August 2018;
- $8.6 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;
- $4.2 million increase in professional services primarily due to continuing accelerated company growth as a result of expansion of our research and development pipeline;
- $5.4 million decrease in pre-clinical expenses primarily due to completion of certain toxicology studies in our PPMO platform;
- $7.2 million decrease in collaboration cost sharing with Summit as it is winding down activities on its Utrophin platform; and
- $8.5 million increase in research and other primarily driven by an increase in lab supplies as a result of an increase in headcount as well as sponsored research with academic institutions.

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- and technology-related 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,			
	2019	2018	Change	Change
	(in thousands)		$	%
Compensation and other personnel expenses	$ 26,992	$ 18,627	$ 8,365	45%
Professional services	25,713	20,547	5,166	25%
Stock-based compensation	13,665	8,224	5,441	66%
Facility- and technology-related expenses	7,167	2,910	4,257	146%
Other	1,892	2,736	(844)	(31)%
Total selling, general and administrative expenses	$ 75,429	$ 53,044	$ 22,385	42%

Selling, general and administrative expenses for the three months ended September 30, 2019 increased by $22.4 million, or 42%, compared with the three months ended September 30, 2018. This was primarily driven by the following:

- $8.4 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $5.2 million increase in professional services primarily due to continuing global expansion;
- $5.4 million increase in stock-based compensation primarily due to increases in headcount and stock price; and
- $4.3 million increase in facility- and technology-related expense primarily due to continuing global expansion offset by a decrease in technology expense due to a change in allocation methodology.

	For the Nine Months Ended September 30,			
	2019	2018	Change	Change
	(in thousands)		$	%
Compensation and other personnel expenses	$ 77,476	$ 49,783	$ 27,693	56%
Professional services	63,193	53,456	9,737	18%
Stock-based compensation	37,556	26,940	10,616	39%
Facility- and technology-related expenses	20,216	12,283	7,933	65%
Restructuring expenses	—	(2,222)	2,222	(100)%
Other	4,947	3,301	1,646	50%
Total selling, general and administrative expenses	$ 203,388	$ 143,541	$ 59,847	42%

Selling, general and administrative expenses for the nine months ended September 30, 2019 increased by $59.8 million, or 42%, compared with the nine months ended September 30, 2018. This was primarily driven by the following:

- $27.7 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $9.7 million increase in professional services primarily due to continuing global expansion;
- $10.6 million increase in stock-based compensation primarily due to increases in headcount and stock price;
- $7.9 million increase in facility- and technology-related expense primarily due to continuing global expansion offset by a decrease in technology expense due to a change in allocation methodology; and
- $2.2 million decrease in restructuring credits due to the relief of cease-use liabilities as a result of the termination of the rental agreement for our Corvallis facility recorded during the second quarter of 2018.

Acquired In-process Research and Development

As a result of the Myonexus acquisition, we recorded acquired in-process research and development expense of approximately $173.2 million during the second quarter of 2019. There was no such transaction during the same period of 2018.

Amortization of In-licensed Rights

Amortization of in-license rights relates to the two license agreements we entered into with BioMarin and UWA in July 2017 and April 2011, respectively. Both in-licensed rights are being amortized on a straight-line basis over the life of the patent from the first commercial sale of EXONDYS 51. For both the three and nine months ended September 30, 2019, and 2018, we recorded amortization of in-licensed rights of approximately $0.2 million and $0.6 million, respectively.

Other expense, net

Other expense, net, primarily consists of interest income on our cash, cash equivalents and investments, interest expense on our debt facilities, amortization of investment discount, and gain from our investment in Lysogene. Our cash equivalents and investments consist of money market funds, commercial paper, government and government agency debt securities, corporate debt securities and certificates of deposit. Interest expense includes interest accrued on our convertible notes, term loan, and revolving line of credit.

For the three and nine months ended September 30, 2019, other expense, net was approximately $2.5 million and $3.5 million, respectively. For the three and nine months ended September 30, 2018, other expense, net was approximately $7.0 million and $16.7 million, respectively. The decrease primarily reflected decreases in term loan termination expenses and an increase in amortization of investment discount as a result of an increase in interest rates.

Income tax expense (benefit)

Income tax expense for the three and nine months ended September 30, 2019 was approximately $0.2 million and $0.5 million, respectively. Income tax (benefit) expense for the three and nine months ended September 30, 2018 was approximately ($0.7) million and $0.1 million, respectively. Income tax expense (benefit) for all periods presented related to state taxes.

Liquidity and Capital Resources

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

	As of September 30, 2019	As of December 31, 2018	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 724,829	$ 370,829	$ 354,000	95%
Short-term investments	324,063	803,083	(479,020)	(60)%
Restricted cash and investments	9,566	1,001	8,565	NM*
Total cash, cash equivalents and investments	$ 1,058,458	$ 1,174,913	$ (116,455)	(10)%
Borrowings:				
Convertible debt	$ 436,421	$ 420,554	$ 15,867	4%
Total borrowings	$ 436,421	$ 420,554	$ 15,867	4%
Working capital				
Current assets	$ 1,362,299	$ 1,426,183	$ (63,884)	(4)%
Current liabilities	215,856	173,690	42,166	24%
Total working capital	$ 1,146,443	$ 1,252,493	$ (106,050)	(8)%

*NM = Not Meaningful

For both the periods ended September 30, 2019 and December 31, 2018, our principal source of liquidity was derived from proceeds from product sales of EXONDYS 51 and equity financings. 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 working capital changes reflect that current assets are lower as a result of reductions in short-term investments, reflecting overall use of cash in operating activities, offset by increases in inventory as we increase inventory to meet our expected requirements. Our current liabilities increase primarily reflects increases in accounts payable due to increased obligations as a result of our operating activities.

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, 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,			
	2019	2018	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ (292,668)	$ (266,240)	$ (26,428)	10%
Investing activities	269,645	(132,752)	402,397	NM*
Financing activities	385,588	8,867	376,721	NM*
Increase (decrease) in cash and cash equivalents	$ 362,565	$ (390,125)	$ 752,690	NM*

*NM = Not Meaningful

Operating Activities.

Cash used in operating activities increased by $26.4 million for the nine months ended September 30, 2019 compared with the nine months ended September 30, 2018, primarily due to the following:

- $85.1 million increase in net loss excluding acquired in-process research and development expense primarily driven by increases in research and development expense and selling, general and administrative expense partially offset by an increase in net product revenues for EXONDYS 51.

The increase of net loss was partially offset by:

- $29.4 net increase in use of operating assets and liabilities; and
- $29.3 million increase in non-cash adjustments.

Investing Activities.

Cash provided by investing activities was $269.6 million for the nine months ended September 30, 2019. Cash used in investing activities for the nine months ended September 30, 2018 was $132.8 million. The favorable change was primarily due to the following:

- $908.3 million increase in proceeds from the sale or maturity of available-for-sale securities.

The increase was partially offset by:

- $172.6 million increase as a result of the acquisition of Myonexus;
- $332.8 million increase in purchase of available-for-sale securities; and
- $1.4 million increase in purchase of property and equipment.

Financing Activities.

Cash provided by financing activities increased by $376.7 million for the nine months ended September 30, 2019 compared with the nine months ended September 30, 2018, primarily driven by the following:

- $365.4 million increase in proceeds from the March 2019 equity financing; and
- $251.9 million decrease in repayment and payment of outstanding debts and debt extinguishment costs.

The increases were partially offset by:

- $217.7 million decrease in proceeds from the revolving line of credit;
- $18.5 million decrease in proceeds from exercise of options and purchase of stock under the Employee Stock Purchase Program; and
- $4.3 million increase in payments related to taxes paid to net share settlement of equity awards

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, 2019:

	Payment Due by Period				
	Total	Less Than 1 Year	1 - 3 Years	3 - 5 Years	More than 5 Years
	(in thousands)				
Convertible debt (1)	$ 613,819	$ 8,550	$ 17,100	$ 17,100	$ 571,069
Lease obligations	70,853	11,613	24,130	22,582	12,528
Manufacturing obligations (2)	850,426	303,965	272,218	114,244	159,999
Total contractual obligations and contingencies	$ 1,535,098	$ 324,128	$ 313,448	$ 153,926	$ 743,596

(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 $1,508.7 million of future development, regulatory, and commercial, and up-front royalty milestone payments associated with our license and collaboration 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, 2019, 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 Quarterly Report on Form 10-Q.

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, 2019, we had approximately $1,058.5 million of cash, cash equivalents and investments, comprised of $724.8 million of cash and cash equivalents, $324.1 million of short-term investments and $9.6 million long-term restricted cash and investments. 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, 2019, 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, 2019, under the supervision and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of the effectiveness of our disclosure controls and procedures pursuant to paragraph (b) of Rules 13a-15 and 15d-15 under the Securities Exchange Act of 1934, as amended (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, 2019, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarterly period ended September 30, 2019, there were no changes in our internal controls over financial reporting that have materially affected or are reasonably likely to materially affect our internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings

For material legal proceedings, please read *Note 14, Commitments and Contingencies - Litigation* to our unaudited condensed consolidated financial statements included in this report.

Item 1A. Risk Factors.

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. and Israel only, although it is available in additional countries through our EAP. 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 generation and dissemination of new data analyses and the consistency of any new data with prior results, whether they support a favorable safety, efficacy and effectiveness 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, ongoing manufacturing efforts 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, effectiveness 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 generation of evidence describing payers, patients and/or societal value of EXONDYS 51;
- whether we can consistently manufacture at acceptable costs;
- the rate and consistency with which EXONDYS 51 is prescribed by physicians, which depends on physicians' views on the safety, effectiveness and efficacy of EXONDYS 51;
- our ability to secure and maintain adequate reimbursement for EXONDYS 51, including the duration of the prior-authorization as well as the number and duration of re-authorization processes required for patients who initially obtained coverage by third parties, including by 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, commercialization or pricing 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 and standards, which could be negatively impacted for various reasons, including 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, failure to meet current Good Manufacturing Practices ("cGMP") requirements 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 Israel; and
- 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.

Even though EXONDYS 51 received accelerated approval by the FDA, 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 the surrogate biomarker of 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 and recordkeeping of EXONDYS 51, and we are required to submit additional safety, efficacy and other post-marketing information to the FDA.

Under the accelerated approval pathway, 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.

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.

We are subject to uncertainty relating to reimbursement policies which, if not favorable, could hinder or prevent the commercial success of EXONDYS 51 and/or our product candidates.

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, and/or we may be required to provide discounts or rebates on EXONDYS 51 in order to obtain or maintain adequate coverage.

We expect that private insurers will continue to consider the efficacy, effectiveness, 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 there are considerable delays in the generation of new evidence or 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 government health programs, private health insurers, including managed care organizations, or other reimbursement 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.

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 list 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, the scrutiny of pharmaceutical pricing, the ongoing debates on reducing government spending and additional legislative proposals. These healthcare reform efforts or any future legislation or regulatory actions aimed at controlling and reducing healthcare costs, including through measures designed to limit reimbursement, restrict access or impose unfavorable pricing modifications on pharmaceutical products, could impact our and our partners' ability to obtain or maintain reimbursement for our products at satisfactory levels, or at all, which could materially harm our business and financial results.

Additionally, our gene therapy product candidates represent novel approaches to treatment that will call for new levels of innovation in both pricing, reimbursement, payment and drug access strategies. Current reimbursement models may not accommodate the unique factors of our gene therapy product candidates, including high up-front costs, lack of long-term efficacy and safety data and fees associated with complex administration, dosing and patient monitoring requirements. Hence, it may be necessary to restructure approaches to payment, pricing strategies and traditional payment models to support these therapies.

The downward pressure on healthcare costs in general has become intense. As a result, increasingly high barriers are being erected to the entry of new products. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be harmed. The manner and level at which reimbursement is provided for services related to our product candidates (e.g., for administration of our product to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and limit our ability to market or sell our products.

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. There are, and may continue to be, judicial challenges. 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 and the introduction of international reference pricing in the U.S. 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 implementation or modification of:

- 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.

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, or if market adoption does not persist, 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 and payor community, including specialists who may purchase or prescribe EXONDYS 51, the clinical efficacy, effectiveness and safety of EXONDYS 51 as the prescription product of choice for 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 ability to timely demonstrate to the satisfaction of payors real world effectiveness and the economic, humanistic and societal benefits of EXONDYS 51;
- 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 (casimersen and golodirsen, respectively), and third parties' competitive therapies.

We may not be able to expand the global footprint of eteplirsen outside of the U.S.

Even though EXONDYS 51 was approved for marketing in the U.S. and in Israel, we may not receive approval to commercialize EXONDYS 51 in additional countries. In November 2016, we submitted a marketing authorization application ("MAA") for eteplirsen to the European Medicines Agency (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. In September 2018, the CHMP of the EMA confirmed its negative opinion for eteplirsen, and the European Commission adopted the CHMP opinion in December 2018. During 2019, we have sought follow-up EMA scientific advice for eteplirsen. Once data from our ongoing studies is available, we plan to evaluate future engagement with the EMA on potential next steps.

In order to market any product in a country outside of the U.S., 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. Obtaining marketing approval in a country outside of the U.S. 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 regulatory authorities outside the U.S. the risk benefit of eteplirsen 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 regulatory authorities outside the U.S.;
- regulatory authorities outside the U.S. 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 outside the U.S. may conclude that data we submit to them fail to demonstrate an appropriate level of safety or efficacy of eteplirsen or that eteplirsen's clinical benefits outweigh its safety risks;
- 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. and Israel 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; and
- regulatory authorities outside the U.S. may adopt new or revised approval policies and regulations.

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. Many foreign countries undertake cost-containment measures that could affect pricing or reimbursement of eteplirsen. 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. In addition, failure to obtain approval in one country or area may affect sales under the EAP in other countries or areas. Even if we are successful in obtaining regulatory approval of eteplirsen in additional countries, our revenue earning capacity will depend on commercial and medical infrastructure, pricing and reimbursement negotiations and decisions with third party payors, including government payors.

We cannot predict whether historical revenues from eteplirsen through our EAP outside the U.S. will continue or whether we will be able to continue to distribute eteplirsen through our EAP.

In certain countries outside the U.S., reimbursement for products that have not yet received marketing authorization may be provided through national EAPs. We have contracted with third party distributors to distribute eteplirsen in certain countries outside the U.S. through EAP, which we plan to expand to other jurisdictions in the future. While we generate revenue from the distribution of eteplirsen through our EAP, we cannot predict whether historical revenues from this program will continue, whether we will be able to continue to distribute our products through EAP in these countries, or whether commercial revenues will exceed revenues historically generated from sales through our EAP. Reimbursement through national EAPs may cease to be available if authorization for an EAP expires or is terminated. For example, healthcare providers in EAP jurisdictions may not be convinced that their patients 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 EAP, the patient will not be able to obtain access to eteplirsen if payment for the drug is not secured.

Any failure to maintain revenues from sales of eteplirsen through our EAP and/or to generate revenues from commercial sales of eteplirsen exceeding historical sales through our EAP could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

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 educating physicians on how 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, efficacy and effectiveness 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.

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. We have built a commercial sales force in Europe and we are currently in the process of building commercial infrastructure 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 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.

If we fail to obtain or maintain regulatory exclusivity for our products, then we may not be able to protect our products from competition and our business may be adversely impacted. If a competitor obtains an authorization to market the same or substantially same product before a product of ours is authorized in a given country and is granted regulatory exclusivity, then our product may not be authorized for sale as a result of the competitor's regulatory exclusivity and as a result, our investment in the development of that product may not be returned.

In addition to any patent protection, we rely on various forms of regulatory exclusivity to protect our products. During the development of our products, we anticipate regulatory exclusivities available upon approval of our products. Implementation and enforcement of regulatory exclusivity, which may consist of regulatory data protection and market protection, varies widely from country to country. Failure to qualify for regulatory exclusivity, or failure to obtain or maintain the extent or duration of such protections that we expect in each of the markets for our products due to challenges, changes or interpretations in the law or otherwise, could affect our revenues for our products or our decision on whether to market our products in a particular country or countries or could otherwise have an adverse impact on our results of operations. We are not guaranteed to receive or maintain regulatory exclusivity for our current or future products, and if our products that are granted orphan status were to lose their status as orphan drugs or the data or marketing exclusivity provided for orphan drugs, our business and operations could be adversely affected.

Due to the nature of EXONDYS 51 and our product candidate pipeline, in addition to new chemical entity exclusivity and new biologic exclusivity, orphan drug exclusivity is especially important for our products that are eligible for orphan drug designation. For eligible products, we plan to rely on orphan drug exclusivity to maintain a competitive position. If we do not have adequate patent protection for our products, then the relative importance of obtaining regulatory exclusivity is even greater. While orphan status for any of our products, if granted or maintained, would provide market exclusivity 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 or similar active ingredient for the same indication during or beyond the exclusivity period applicable to our product on the basis of orphan drug status (e.g., seven years in the U.S.). Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process.

In addition, we may face risks with maintaining regulatory exclusivities for our products, and our protection may be circumvented, even if maintained. For instance, orphan drug exclusivity in the U.S. may be rescinded if (i) an alternative, competing product demonstrates clinical superiority to our product with orphan exclusivity; or (ii) we are unable to assure the availability of sufficient quantities of our orphan product to meet the needs of patients. Moreover, competitors may receive approval of different drugs or biologics for indications for which our prior approved orphan product has exclusivity. Orphan drug exclusivity in Europe 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. Thus, we cannot guarantee that another company will not receive approval to market a product candidate that is granted orphan drug exclusivity for the same drug or similar drug and same orphan indication as any of our product candidates for which we plan to file an NDA, Biologics License Application ("BLA") or MAA. If that were to happen, our prior approved orphan product may face competition and any pending NDA, BLA 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.

Even though we have obtained orphan drug designation for certain of our product candidates and even if we obtain orphan drug designation for these or our future product candidates, due to the uncertainties associated with developing biopharmaceutical products, we may not be the first to obtain marketing approval for any particular orphan indication, which means that we may not obtain orphan drug exclusivity and could also potentially be blocked from approval of certain product candidates until the competitor's orphan drug exclusivity period on its product expires (e.g., seven years in the U.S.). Moreover, with respect to antisense oligonucleotides and gene therapies, it is uncertain how similarity between product candidates designed to treat the same rare disease or condition may be determined on a country-by-country basis and whether the orphan drug exclusivity of a previously approved product can block the approval of a chemically distinct product candidate under regulatory review.

The patient population suffering from DMD, LGMDs, Pompe disease, CMT 1A 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, CMT 1A and MPS IIIA are rare, fatal genetic 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. CMT is a group of peripheral nerve disorders affecting approximately one in every 2,500 individuals. CMT type 1A affects approximately 50,000 patients in the U.S. MPS IIIA affects approximately 1 in 100,000 newborns. Our estimates of the size of these patient populations are based on limited number of published studies as well as internal analyses. Various factors may decrease the market size of our product and product candidates, including the severity of the disease, patient demographics and the response of patients' immune systems to our product candidates. If the results of these studies or our analysis of them do not accurately reflect the relevant patient population, 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.

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 in which our product and product candidates are aimed. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with our product or our product candidates. For example, we face competition in the field of DMD by third parties who are developing or who had once developed: (i) exon skipping product candidates, such as Wave Life Sciences (notably for exons 51 and 53), Nippon Shinyaku (notably for exon 53), Daiichi Sankyo (notably for exon 45) and Audentes Therapeutics, Inc. (notably for exons 51 and 53); (ii) gene therapies that express microdystrophin or mini-dystrophin, such as Pfizer and Solid Biosciences; (iii) CRISPR/Cas 9 approaches, such as Exonics Therapeutics (acquired by Vertex Pharmaceuticals), CRISPR Therapeutics and Editas Medicine; (iv) other disease modifying approaches, such as PTC Therapeutics, which has a small molecule candidate, ataluren, that targets nonsense mutations; and (v) other approaches that may be palliative in nature or potentially complementary with our product and product candidates and that are being developed by Santhera, Catabasis, Fibrogen, ReveraGen, Capricor and Tivorsan. 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.

In addition, we are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development using platform technologies that may be viewed as competing with ours beyond and including those companies mentioned immediately above, such as Alnylam Pharmaceuticals, Inc., Ionis Pharmaceuticals, Inc., Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Shire plc (now Takeda), Biogen and Sanofi. 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 (now Takeda), Eli Lilly, Alnylam Pharmaceuticals, Inc., Moderna Therapeutics, Inc., Summit, Akashi, Catabasis, Capricor Therapeutics, Oxford University, Exonics Therapeutics (acquired by Vertex Pharmaceuticals), and Editas Medicine.

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

It is possible that our competitors will succeed in developing technologies that limit the market size for our product or product candidates, impact the regulatory approval and post-marketing process for our product and product candidates, are more effective than our product candidates or 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 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.

In order to achieve our long-term business objectives, we actively evaluate various strategic transactions on an ongoing basis, including licensing or acquiring products, technologies or businesses. We may face competition from other companies in pursuing acquisitions and similar transactions in the biotechnology industry. This competition is most intense for approved drugs and late-stage drug candidates, which have the lowest risk and would have the most immediate effect on our financial performance. 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, we may not realize the anticipated benefits of such transaction, each of which involves numerous risks, including:

- failure to successfully develop the acquired or licensed drugs or technology or to achieve strategic objectives, including successfully developing and commercializing the drugs, drug candidates or technologies that we acquire or license;
- entry into markets in which we have no or limited direct prior experience or where competitors in such markets have stronger market positions;
- disruption of our ongoing business, distraction of our management and employees from other opportunities and challenges and retention of key employees;
- potential failure of the due diligence processes to identify significant problems, liabilities or other shortcomings or challenges of an acquired company, or acquired or licensed product or technology, including but not limited to, problems, liabilities or other shortcomings or challenges with respect to intellectual property, product quality, safety, accounting practices, employee, customer or third party relations and other known and unknown liabilities;
- liability for activities of the acquired company or licensor before the acquisition or license, including intellectual property infringement claims, violations of laws, commercial disputes, tax liabilities, and other known and unknown liabilities;
- exposure to litigation or other claims in connection with, or inheritance of claims or litigation risk as a result of an acquisition or license, including but not limited to, claims from terminated employees, customers, former equity holders or other third-parties;
- difficulty in integrating the drugs, drug candidates, technologies, business operations and personnel of an acquired asset or company; and
- difficulties in the integration of the acquired company's departments, systems, including accounting, human resource and other administrative systems, technologies, books and records, and procedures, as well as in maintaining uniform standards, controls, including internal control over financial reporting required by the Sarbanes-Oxley Act of 2002 and related procedures and policies.

In addition, even if we achieve the long-term benefits associated with strategic transactions, our expenses and short-term costs may increase materially and adversely affect our liquidity and short-term net income (loss). Future licenses or acquisitions could result in potentially dilutive issuances of equity securities, the incurrence of debt, the creation of contingent liabilities, impairment expenses related to goodwill, and impairment or amortization expenses related to other intangible assets, which could harm our financial condition.

Risks Related to the Development of our Product Candidates

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on the speed at which we can recruit eligible patients to participate in testing our product candidates. We have experienced delays in some of our clinical trials, and we may experience similar delays in the future. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology or termination of the clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics to achieve diversity in a study, to complete our clinical trials in a timely manner. Patient enrollment is affected by factors including:

- design of the study protocol;
- size of the patient population;
- eligibility criteria for the study in question;
- manufacturing of product candidates;
- perceived risks and benefits of the product candidate under study, including as a result of adverse effects observed in similar or competing therapies;
- proximity and availability of clinical trial sites for prospective patients;

- availability of competing therapies and clinical trials;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- activities of patient advocacy groups;
- ability to monitor patients adequately during and after treatment; and
- severity of the disease under investigation.

In particular, each of the conditions for which we plan to evaluate our product candidates are rare genetic diseases with limited patient pools from which to draw for clinical trials. Further, because newborn screening for these diseases is not widely adopted, and it can be difficult to diagnose these diseases in the absence of a genetic screen, we may have difficulty finding patients who are eligible to participate in our studies. The eligibility criteria of our clinical trials will further limit the pool of available study participants. Additionally, the process of finding and diagnosing patients may prove costly. The treating physicians in our clinical trials may also use their medical discretion in advising patients enrolled in our clinical trials to withdraw from our studies to try alternative therapies.

We may not be able to initiate or continue clinical trials if we cannot enroll a sufficient number of eligible patients to participate in the clinical trials required by the FDA or the EMA or other regulatory agencies. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with contract research organizations ("CROs") and physicians;
- different standards for the conduct of clinical trials;
- our inability to locate qualified local consultants, physicians and partners; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business.

Failures or delays in the commencement or completion of ongoing and planned clinical trials of our product candidates could cause us not to meet our expected timelines or result in increased costs to us, and could delay, prevent or limit our ability to gain regulatory approval of any product candidate and to generate revenue and continue our business.

Successful completion of clinical trials at each applicable stage of development is a prerequisite to submitting a marketing application to the regulatory agencies and, consequently, the ultimate approval and commercial marketing of any of our product candidates for the indications in which we develop them. We do not know whether any of our clinical trials will begin or be completed, and results announced, as planned or expected, if at all, as the commencement and completion of clinical trials and announcement of results can be delayed or prevented for a number of reasons, including, among others:

- denial by the regulatory agencies of permission to proceed with our planned clinical trials or any other clinical trials we may initiate, or placement of a clinical trial on hold;
- delays in filing or receiving approvals of additional INDs that may be required;
- negative results from our ongoing non-clinical trials or clinical trials;
- challenges in identifying, recruiting, enrolling and retaining patients to participate in clinical trials
- 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;

- inadequate quantity or quality of supplies of a product candidate or other materials necessary to conduct clinical trials, for example as a result of delays in defining and implementing the manufacturing process for materials used in pivotal trials or for the manufacture of larger quantities or other delays or issues arising in the manufacturing of sufficient supply of finished drug product;
- difficulties obtaining institutional review board ("IRB") approval, and equivalent approval for sites outside the U.S., to conduct a clinical trial at a prospective site or sites;
- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;
- delays or problems in analyzing data, or the need for additional analysis or data or the need to enroll additional patients;
- the occurrence of serious adverse events or unexpected drug-related side effects experienced by patients in a clinical trial or unexpected results in ongoing non-clinical trials;
- delays in validating endpoints utilized in a clinical trial;
- our inability to satisfy the requirements of the regulatory agencies to commence clinical trials, including CMC requirements, or other regulatory requirements prior to the initiation of a clinical trial;
- the regulatory agencies disagreeing with our clinical trial design and our interpretation of data from clinical trials, or changing the requirements for approval even after the regulatory authority has reviewed and commented on the design for our clinical trials;
- reports from non-clinical or clinical testing of competing therapies that raise safety or efficacy concerns; and
- the recruitment and retention of employees, consultants or contractors with the required level of expertise.

Any inability to successfully complete pre-clinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to demonstrate comparability of our modified product candidates to earlier versions. Clinical study delays could also shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business and results of operations.

Results from pre-clinical and early-stage clinical trials may not be indicative of efficacy in late-stage clinical trials, and pre-clinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, which could prevent or significantly delay their regulatory approval.

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive pre-clinical and clinical 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 we believe the pre-clinical data for PPMO SRP-5051 collected to date is positive, 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. Some of our clinical trials were conducted with small patient populations and were not blinded or placebo-controlled, making it difficult to predict whether the favorable results that we observed in such trials will be repeated in larger and more advanced clinical trials. For example, on October 3, 2018, Nationwide presented positive results from a Phase 1/2a micro-dystrophin gene therapy clinical trial in four individuals with DMD enrolled in the trial and, on March 25, 2019, we presented nine-month functional and creatine kinase ("CK") data from baseline from these four individuals, and twelve-months CK data from baseline from one of these individuals. In addition, on February 27, 2019, we announced positive expression and biomarker data from the first three-patient cohort dosed in the MYO-101 gene therapy trial to treat LGMD type 2E, or beta-sarcoglycanopathy and, on October 4, 2019, we announced positive nine-month data from these three patients. The data is based on small patient samples and therefore may not be predictive of future results. In addition, we cannot assure that the results of additional data or data from any future trial will yield results that are consistent with the data presented, that we will be able to demonstrate the safety and efficacy of these product candidates, 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 any of such product candidates. Similarly, we cannot provide assurances that data from our studies with respect to EXONDYS 51, golodirsen, casimersen, gene therapy-based product candidates and other 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.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent regulatory approval of product candidates, limit the commercial potential or result in significant negative consequences following any potential marketing approval.

Our product candidates may cause undesirable side effects. In addition to side effects caused by product candidates, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur in our trials, we may decide, or the FDA, the EMA or other regulatory authorities could order us, to halt, delay or amend pre-clinical development or clinical development of our product candidates or we may be unable to receive regulatory approval of our product candidates for any or all targeted indications. Even if we are able to demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates and may harm our business, financial condition and prospects significantly.

Our gene therapy product candidates may be perceived as unsafe or may result in unforeseen adverse events. Failure of other gene therapy programs, negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our gene therapy product candidates and harm our ability to conduct our business or obtain regulatory approvals for our gene therapy product candidates.

Gene therapy remains a newly applied technology, with only a few gene therapy products approved to date in the U.S., the EU or elsewhere. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available.

In addition, ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed their intentions to further regulate biotechnology. More restrictive regulations or claims that our product candidates are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

More restrictive government regulations or negative public opinion would harm our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our gene therapy product candidates or demand for any products we may develop. For example, earlier gene therapy trials led to several well-publicized adverse events, including death. Lack of efficacy and/or serious adverse events related to clinical trials we, our strategic partners or other companies conduct, even if such adverse events are not ultimately attributable to the relevant product candidates or products, and/or failed commercialization of gene therapy products may result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

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. Of the large number of drugs in development in the biopharmaceutical industry, only a small percentage result in the submission of a marketing application to the FDA or an MAA to the EMA and even fewer are approved for commercialization.

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, BLA or MAA submissions for one or more of our product candidates, and may delay, reject or refuse to accept for review, or approve any 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 or BLA we submit to the FDA. Even if we meet FDA requirements and an advisory committee votes to recommend approval of an NDA or BLA 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, gene therapy and other 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. 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, BLAs 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, dystrophin analyses and using different assays), 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 BLA or result in a decision by the Company not to proceed with an NDA or BLA 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, BLA or MAA submissions.

Even if our product candidates demonstrate safety and efficacy in clinical studies, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory advisory group or authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical studies and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. Finally, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates. 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 or any of our strategic partners are unable to develop, or obtain regulatory approval for, or, if approved, maintain regulatory compliance and successfully commercialize, our product candidates, our business will be materially harmed.

We are investing significant resources in the development of novel gene therapy product candidates. Only a few gene therapy products have been approved in the U.S. and EU. If we are unable to show the safety and efficacy of these product candidates, experience delays in doing so or are unable to successfully commercialize at least one of these drugs, our business would be materially harmed.

We are investing significant resources in the development of our gene therapy product candidates. We believe that a significant portion of the long-term value attributed to our company by investors is based on the commercial potential of these product candidates. There can be no assurance that any development problems we experience in the future related to our gene therapy programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. Initial results from ongoing clinical trials may differ materially from final results from such clinical trials. The results from pre-clinical and early clinical studies do not always accurately predict results in later, large-scale clinical trials. We may also experience delays in developing a sustainable, reproducible and commercial-scale manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical trials or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Currently, only a few gene therapy products have been approved in the Western world. Novartis's and Gilead's CAR-T therapies both received approval from the FDA in 2017. Given the few precedents of approved gene therapy products, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our gene therapy product candidates in the U.S., the EU or other jurisdictions. Approvals by the EMA and the European Commission may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene therapy products have evolved and may continue to change in the future. For example, on July 11, 2018, the FDA released draft guidance documents intended to reflect recent advances in the field, and to update the framework for the development, review and approval of gene therapies. These draft guidance documents pertain to the development of gene therapies for the treatment of specific disease categories, including rare diseases, and to manufacturing and long-term follow up issues relevant to gene therapy, among other topics. Furthermore, the FDA has established the Office of Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the U.S. National Institutes of Health ("NIH"), are also subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee ("RAC"). Although the FDA decides whether individual gene therapy protocols may proceed, the RAC review process can impede the initiation of a clinical trial, even if the FDA has reviewed the study and approved its initiation. Clinical trial sites in the U.S. that receive NIH funding for research involving recombinant or synthetic nucleic acid molecules are required to follow RAC recommendations, or risk losing NIH funding for such research or needing NIH pre-approval before conducting such research. In addition, the FDA can put an IND, on clinical hold if the information in an IND is not sufficient to assess the risks in pediatric patients. Before a clinical trial can begin at any institution, that institution's IRB, and its Institutional Biosafety Committee will have to review the proposed clinical trial to assess the safety of the study. Moreover, serious adverse events or developments in clinical trials of gene therapy product candidates conducted by others may cause the FDA or other regulatory bodies to initiate a clinical hold on our clinical trials or otherwise change the requirements for approval of any of our product candidates.

These regulatory review agencies, committees and advisory groups and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval studies, limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates.

If the anticipated or actual timing of marketing approvals for our gene therapy product candidates, or the market acceptance of these product candidates, if approved, including treatment reimbursement levels agreed to by third-party payors, do not meet the expectations of investors or public market analysts, the market price of our common stock would likely decline.

Because we are developing product candidates for the treatment of certain diseases in which there is little clinical experience and we are using new endpoints or methodologies, there is increased risk that the FDA, the EMA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze.

During the FDA review process, we will need to identify success criteria and endpoints such that the FDA will be able to determine the clinical efficacy and safety profile of our product candidates. As we are developing novel treatments for diseases in which there is little clinical experience with new endpoints and methodologies, such as gene therapy, there is heightened risk that the FDA, the EMA or other regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance. 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 full or accelerated regulatory approval.

If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, 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.

Fast track product, breakthrough therapy, priority review, or Regenerative Medicine Advanced Therapy ("RMAT") designation by the FDA, or access to the PRIME scheme by the EMA, for our product candidates may not lead to faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek fast track, breakthrough therapy designation, RMAT designation, PRIME scheme access or priority review designation for our product candidates if supported by the results of clinical trials. A fast track product designation is designed to facilitate the clinical development and expedite the review of drugs intended to treat a serious or life-threatening condition which demonstrate the potential to address an unmet medical need. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. A RMAT designation is designed to accelerate approval for regenerative advanced therapies such as our gene therapy product candidates. Priority review designation is intended to speed the FDA marketing application review timeframe for drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. PRIME is a scheme provided by the EMA to enhance support for the development of medicines that target an unmet medical need.

For drugs and biologics that have been designated as fast track products or breakthrough therapies, or granted access to the PRIME schema, interaction and communication between the regulatory agency and the sponsor of the trial can help to identify the most efficient path for clinical development. Sponsors of drugs with fast track products or breakthrough therapies may also be able to submit marketing applications on a rolling basis, meaning that the FDA may review portions of a marketing application before the sponsor submits the complete application to the FDA, if the sponsor pays the user fee upon submission of the first portion of the marketing application. For products that receive a priority review designation, the FDA's marketing application review goal is shortened to six months, as opposed to ten months under standard review. This review goal is based on the date the FDA accepts the marketing application for review, this application validation period typically adds approximately two months to the timeline for review and decision from the date of submission. RMAT designations will accelerate approval but the exact mechanisms have not yet been announced by FDA.

Designation as a fast track product, breakthrough therapy, RMAT, PRIME, or priority review product is within the discretion of the regulatory agency. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a fast track product, breakthrough therapy, RMAT, PRIME, or priority review product, the agency may disagree and instead determine not to make such designation. In any event, the receipt of such a designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional regulatory procedures and does not assure ultimate marketing approval by the agency. In addition, regarding fast track products and breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification as either a fast track product, RMAT, or a breakthrough therapy or, for priority review products, decide that period for FDA review or approval will not be shortened.

We may not be able to advance all of our programs, and we may use our financial and human resources to pursue particular programs and fail to capitalize on programs that may be more profitable or for which there is a greater likelihood of success.

Our pipeline includes more than 20 programs in various stages of development for a broad range of diseases and disorders. We plan to expand our pipeline through internal research and development and through strategic transactions. Because we have limited resources, we may not be able to advance all of our programs. We may also forego or delay pursuit of opportunities with certain programs or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

Risks Related to Third Parties

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 through our EAP. 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.

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 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 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.

Our reliance on third parties requires us to share our proprietary information, which increases the possibility that a competitor will discover them or that our proprietary information will be misappropriated or inadvertently disclosed.

The use of third-party service providers requires us to disclose our proprietary information to these parties, which could increase the risk that a competitor will discover this information or that this information will be misappropriated or disclosed without our intent to do so. 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.

Risks Related to Manufacturing

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, EAP, 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 early access programs demand for eteplirsen, or to conduct our research and development programs and conduct clinical trials for our product candidates. Therefore, we rely on, and expect to continue relying on for the foreseeable future, a limited number of third parties to manufacture and supply materials (including raw materials and subunits), API and drug product, as well as to perform additional steps in the manufacturing process, such as labeling and packaging of vials and storage of EXONDYS 51 and our product candidates. There are a limited number of third parties with facilities and capabilities suited for the manufacturing process of EXONDYS 51 and our product candidates, which creates a heightened risk that we may not be able to obtain materials and APIs in the quantity and purity that we require.

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

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

Furthermore, any problems in our manufacturing process or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in third-party manufacturing processes or facilities also could restrict our ability to meet market demand for our products.

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, early access programs 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. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and /or substantial termination penalties, which could have a material adverse effect on our business prior to and after commercialization.

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

Our contract manufacturers are required to produce our materials, APIs and drug products under cGMP. We and our contract manufacturers are subject to periodic inspections by the FDA, EMA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations. In addition, before we can begin to commercially manufacture our product candidates in third-party or our own facilities, we must obtain regulatory approval from the FDA, which includes a review of the manufacturing process and facility. A manufacturing authorization must also be obtained from the appropriate European Union regulatory authorities and may be required by other foreign regulatory authorities. The timeframe required to obtain such approval or authorization is uncertain. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements.

While we work diligently with all contract manufacturers to maintain full compliance via routine audit programs, we do not have direct operational 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. For example, we were notified by the Research Institute at Nationwide 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 Good Manufacturing Practices (“GMP”) 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.

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 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.

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 and other product candidates 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 other 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 may be delayed or otherwise negatively impacted, which could significantly harm our business.

During our 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. 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 property rights required for the manufacturing process needed for large-scale clinical trials or commercialization of EXONDYS 51 or the continued development of our product candidates could cause significant delays in our business plans or otherwise negatively impact the commercialization of EXONDYS 51 or the continued development of our product candidates.

Products intended for use in gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization of gene therapy programs, limit the supply of our products or otherwise harm our business.

We currently have development, manufacturing and testing agreements with third parties to manufacture supplies of our gene therapy product candidates. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of suppliers.

The physical and chemical properties of biologics such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we employ multiple steps to control our manufacturing process to assure that the process works and the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical and/or commercial-grade materials that meet FDA, EMA or other applicable foreign standards or specifications with consistent and acceptable production yields and costs.

In addition, the FDA, the EMA and other foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA or other foreign regulatory authorities may require that we not distribute a lot until the competent authority authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability and deviations among different sites, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay clinical trials or product launches which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to operate our manufacturing process which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

Furthermore, no manufacturer currently has the experience or ability to produce our vectors or gene therapy product candidates at commercial levels. Even if we timely develop a manufacturing process and successfully transfer it to the third-party vector and product manufacturers or successfully and timely develop our internal capacity, if we or such third-party manufacturers are unable to produce the necessary quantities of viral vectors and our product candidates, or in compliance with GMP or other pertinent regulatory requirements, and within our planned time frame and cost parameters, it may result in delays in our development plans or increased capital expenditures, and the development and sales of our products, if approved, may be materially harmed.

Risks Related to our Intellectual Property

Our success, competitive position and future revenue depend in part on our ability and the abilities of our licensors and other collaborators to obtain, maintain and defend the patent protection for our product, product candidates, and platform technologies, to preserve our trade secrets, and to prevent third parties from infringing on our proprietary rights.

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 product, product candidates and platform technologies. We anticipate filing additional patent applications both in the U.S. and in other countries. Our success will depend, in significant part, on our ability to obtain, maintain and defend our U.S. and foreign patents covering our product, product candidates and platform technologies as well as preserving our trade secrets for these assets. The patent process is subject to numerous risks and uncertainties, and we can provide no assurance that we will be successful in obtaining, maintaining, or defending our patents. Even when our patent claims are allowed, the claims may not issue, or in the event of issuance, may not be sufficient to protect our product, product candidates or platform technologies.

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. 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. Thus, 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.

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, particularly as a tactic to impose a price control. 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.

We may be able to assert that certain activities engaged in by our competitors 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. We may not be able to successfully defend patents necessary to prevent competitors from commercializing competing product candidates. Our patent rights might be challenged, invalidated, circumvented or otherwise not provide any competitive advantage. Defending our patent positions may require significant financial resources and could negatively impact other Company objectives.

Under the Hatch-Waxman Act, one or more motivated third parties may file an Abbreviated New Drug Application, seeking approval of a generic copy of an innovator product approved under the NDA pathway such as our PMO product, or a New Drug Application under Section 505(b)(2), which may be for a new or improved version of the original innovator product. The third parties are allowed to rely on the safety and efficacy data of the innovator's product, may not need to conduct clinical trials and can market a competing version of a product after the expiration or loss of patent exclusivity or the expiration or loss of regulatory exclusivity and often charge significantly lower prices. Upon the expiration or loss of patent protection or the expiration or loss of regulatory exclusivity for a product, the major portion of revenues for that product may be dramatically reduced in a very short period of time. If we are not successful in defending our patents and regulatory exclusivities, we will not derive the expected benefit from them.

The DMD patent landscape is continually evolving, and we may be able to assert that certain activities engaged in by third parties infringe 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.

Uncertainty over intellectual property in the pharmaceutical and biotechnology industry has been the source of litigation and other disputes, which is inherently costly and unpredictable.

Litigation, interferences, oppositions, inter partes reviews, administrative challenges or other similar types of proceedings are, have been and may in the future be necessary in some instances to determine the validity and scope of certain of our proprietary rights, and in other instances to determine the validity, scope or non-infringement of certain patent rights claimed by third parties to be pertinent to the manufacture, use or sale of our products. We may also face challenges to our patent and regulatory exclusivities covering our products by third parties, including manufacturers of generics and biosimilars that may choose to launch or attempt to launch their products before the expiration of our patent or regulatory exclusivity. Litigation, interferences, oppositions, inter partes reviews, administrative challenges or other similar types of proceedings are unpredictable and may be protracted, expensive and distracting to management. The outcomes of such proceedings could adversely affect the validity and scope of our patents or other proprietary rights, hinder our ability to manufacture and market our products, require us to seek a license for the infringed products or technology or result in the assessment of significant monetary damages against us that may exceed amounts, if any, accrued in our financial statements. An adverse determination in a judicial or administrative proceeding or a failure to obtain necessary licenses could prevent us from manufacturing or selling our products. Furthermore, payments under any licenses that we are able to obtain would reduce our profits derived from our products. Any of these circumstances could result in financial, business or reputational harm to us or could cause a decline or volatility in our stock price.

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 Patent Trial and Appeal Board ("PTAB") seeking to challenge the validity of some or all of the claims in any of our patents through an inter partes review or other post-grant proceeding. Should the PTAB institute an inter partes review 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.

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

Similar to us, competitors continually seek intellectual property protection for their technology. Several of our development programs, particularly gene therapy programs, focus on therapeutic areas that have been the subject of extensive research and development by third parties for many years and have been protected with third party patent rights. Due to the amount of intellectual property in our various fields of technology, we cannot be certain that we do not infringe intellectual property rights of competitors or that we will not infringe intellectual property rights of competitors granted or created in the future. Our competitors might have obtained, or could obtain in the future, patents that limit, interfere with or eliminate our ability to make, use and sell our product, product candidates or platform technologies in important commercial markets.

In order to maintain or obtain freedom to operate for our products and product candidates, we may incur significant expenses, including those associated with entering into agreements with third parties that require milestone and royalty payments. Additionally, if we were to challenge the patent rights of our competitors, we might not be successful.

If our product, product candidates, or platform 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, or cease commercialization of an infringing product;
- redesign our product, 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 result in product and product candidate development delays or cessation, and as such substantially harm our potential earnings, financial condition and operations. The patent landscape of our product candidates 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 our product, product candidates or platform technologies infringe on the intellectual property rights of such parties. 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.

Risks Related to our Business Operations

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, within the United States, certain federal and state healthcare laws and regulations will apply to or affect our business. The laws and 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.

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. 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.

In connection with the commercial launch of EXONDYS 51, we have enhanced 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. If our operations are found to be in violation of any of the laws described above or any other laws, rules or regulations that apply to us, we will be subject to penalties, including civil and criminal penalties, damages, fines and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could adversely affect our ability to operate our business and our financial results. 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. Even if we successfully defend against an action against us for violation of a law, the action and our defense could nonetheless cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security, fraud and reporting laws may prove costly.

We 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 commercial general liability insurance coverage for our clinical trials and 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.

If we, our collaborators, or any third-party manufacturers engaged by us or our collaborators fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We, our collaborators, and any third-party manufacturers we engage are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of bio-hazardous materials. Our operations involve the use of hazardous materials, including organic and inorganic solvents and reagents. 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.

Further, with respect to the operations of any current or future collaborators or third party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our product or product candidates, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product or product candidates.

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.

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 gene therapy 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.

We expect to expand our organization and may experience difficulties in managing this growth, which could disrupt our operations.

As our business activities expand, we expect to expand our full-time employee base and to hire more consultants and contractors. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. 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 effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy.

Our sales and operations are subject to the risks of doing business internationally.

We are increasing our presence in international markets, including emerging markets, subjecting us to many risks that could adversely affect our business and revenues, such as:

- the inability to obtain necessary foreign regulatory or pricing approvals of products in a timely manner;
- uncertainties regarding the collectability of accounts receivable;
- fluctuations in foreign currency exchange rates that may adversely impact our revenues, net income and value of certain of our investments;
- difficulties in staffing and managing international operations;
- the imposition of governmental controls;
- less favorable intellectual property or other applicable laws;

- increasingly complex standards for complying with foreign laws and regulations that may differ substantially from country to country and may conflict with corresponding U.S. laws and regulations;
- the far-reaching anti-bribery and anti-corruption legislation in the U.K., including the U.K. Bribery Act 2010, and elsewhere and escalation of investigations and prosecutions pursuant to such laws;
- compliance with complex import and export control laws;
- restrictions on direct investments by foreign entities and trade restrictions; and
- changes in tax laws and tariffs.

In addition, our international operations are subject to regulation under U.S. law. For example, the Foreign Corrupt Practices Act (FCPA) prohibits U.S. companies and their representatives from paying, offering to pay, promising to pay or authorizing the payment of anything of value to any foreign government official, government staff member, political party or political candidate for the purpose of obtaining or retaining business or to otherwise obtain favorable treatment or influence a person working in an official capacity. In many countries, the health care professionals we regularly interact with may meet the FCPA's definition of a foreign government official. Failure to comply with domestic or foreign laws could result in various adverse consequences, including: possible delay in approval or refusal to approve a product, recalls, seizures or withdrawal of an approved product from the market, disruption in the supply or availability of our products or suspension of export or import privileges, the imposition of civil or criminal sanctions, the prosecution of executives overseeing our international operations and damage to our reputation. Any significant impairment of our ability to sell products outside of the U.S. could adversely impact our business and financial results.

Unfavorable global economic conditions could harm our business, financial condition or results of operations.

Our results of operations could be harmed by general conditions in the global economy and in the global financial markets. A severe or prolonged economic downturn could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. For example, the global financial crisis of 2007-2008 and the ongoing European economic crisis caused extreme volatility and disruptions in the capital and credit markets. A weak or declining economy could strain our manufacturers, possibly resulting in manufacturing disruption, or cause delays in payments for our services by third-party payors or our future collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could harm our business.

Additionally, in June 2016, a majority of United Kingdom (UK) voters voted for the UK to exit the European Union (Brexit) and in March 2017, the UK government provided official legal notification to the European Union that the UK will exit the European Union. The timing and completion of Brexit is subject to judicial and parliamentary developments in the UK, as well as any legal challenges. The economic effects of Brexit will depend on any agreements the UK makes to retain access to European Union markets either during a transitional period or more permanently. Brexit could adversely affect European and worldwide economic or market conditions and could contribute to instability in global financial markets. Brexit is likely to lead to legal uncertainty and potentially divergent national laws and regulations as the UK determines which European Union laws to replace or replicate. Any of these effects of Brexit, and any other effects we cannot anticipate, could adversely affect our business, business opportunities, results of operations, financial condition and cash flows.

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.

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.

We continue to evaluate the overall impact of the TCJA on our business. There is continued uncertainty in the TCJA, and although changes or challenges cannot be predicted, we believe we have used reasonable assumptions and interpretations in applying TCJA. We continue to monitor for legislative developments, issuance of regulations and technical memorandum to provide further clarification and/or interpretations of the TCJA and will adjust our financial statements as needed.

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.

We are winding down our expired U.S. government contracts, and the U.S. government may deny payment of some or all of the currently outstanding amounts owed to us. In addition, further development of our infectious disease programs may be limited 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 infectious disease development programs. These contracts are expired and we are currently involved in contract close-out activities. The U.S. government has the right to perform additional audits prior to making final payment of costs and fees. If we are not able to adequately support costs incurred or other government requirements, the government may deny payment of 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.

Our employees, principal investigators, consultants and strategic partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and strategic partners. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. We adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

The increasing use of social media platforms presents new risks and challenges.

Social media is increasingly being used to communicate about our product, technologies and programs, and the diseases our product and product candidates are designed to treat. Social media practices in the biopharmaceutical industry continue to evolve and regulations relating to such use are not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media channels to comment on the effectiveness of a product or to report an alleged adverse event. When such disclosures occur, there is a risk that we fail to monitor and comply with applicable adverse event reporting obligations or we may not be able to defend the Company or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our product and/or product candidates. There is also a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us on any social networking website. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face overly restrictive regulatory actions or incur other harm to our business.

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our office, manufacturing and/or lab spaces, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

Risks Related to our Financial Condition and Capital Requirements

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

We incurred an operating loss of $123.6 million and $475.3 million for the three and nine months ended September 30, 2019, respectively. Our accumulated deficit was $2.1 billion as of September 30, 2019. 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. Since EXONDYS 51 and our product candidates target small patient populations, 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 generally incurred expenses related to research and development of our technologies and product candidates and 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 2019. 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 to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

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 to continue the development of product candidates in our pipeline, to prepare for potential commercialization of our product candidates, 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.

While we are currently well capitalized, we may use available capital resources sooner than we expect under our current operating plan. In addition, our operating plan may change. We may need or choose to seek additional funds sooner than planned, through equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. In any event, we expect to require additional capital to expand future development efforts, obtain regulatory approval for, and to commercialize, our product candidates. Raising funds in the current economic environment may present additional challenges. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or in light of specific strategic considerations.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. In the event we receive negative data from our key clinical programs or encounter other major setbacks in our development, manufacturing or regulatory activities or in our commercialization efforts, our stock price is likely to decline, which would make a future financing more difficult. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders. The issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners 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.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product, if approved, or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights.

We may seek additional capital through a combination of private and public equity offerings, debt financings, collaborations and strategic and licensing arrangements. To the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, the ownership interest of our stockholders in our company will be diluted. In addition, the terms of any such securities may include liquidation or other preferences that materially adversely affect the rights of our stockholders. Debt financing, if available, would increase our fixed payment obligations and 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. If we raise additional funds through collaboration, strategic partnerships and licensing arrangements with third parties, we may have to relinquish valuable rights to our product candidates, our intellectual property, future revenue streams or grant licenses on terms that are not 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.

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-six months, our stock has increased as much as 37% in a single day or decreased as much as 15% 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.

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.

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.

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 to attract and retain employees, directors and consultants. We may also issue shares of common stock to finance our operations and in connection with our strategic goals. 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.

Currently, our Amended and Restated Certificate of Incorporation authorizes the issuance of up to 99.0 million shares of common stock. As of September 30, 2019, there were approximately 74.5 million shares of common stock outstanding and outstanding awards to purchase 9.6 million shares of common stock under various incentive stock plans. Additionally, as of September 30, 2019, there were approximately 3.4 million shares of common stock available for future issuance under our 2018 Equity Incentive Plan, approximately 0.6 million shares of common stock available for issuance under our Amended and Restated 2013 Employee Stock Purchase Plan, and approximately 0.7 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan.

We may issue additional shares to grant equity awards 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. We may also issue additional common stock and warrants from time to time to finance our operations and in connection with strategic transactions, such as acquisitions and licensing. We may need to increase our authorized shares of common stock under our Amended and Restated Certificate of Incorporation to support these strategic goals. There can be no assurance that we will be able to obtain shareholder approval to increase the number of authorized shares.

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.

Future sales of our common stock in the public market could cause our share price to fall.

Sales of a substantial number of our common stock in the public market, including sales by members of our management or board of directors, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity or equity-related securities.

Risks Related to Our Convertible Senior Notes

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

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

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

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

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

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

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

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

None.

Item 6. Exhibits.

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

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
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	Inline eXtensible Business Reporting Language (XBRL) Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.					X
101.SCH	Inline XBRL Taxonomy Extension Schema Document.					X
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document.					X
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)					X

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

* Identified information has been excluded from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

** 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: November 7, 2019

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

Date: November 7, 2019

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

EXHIBIT 31.1

CERTIFICATION

I, Douglas S. Ingram, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Sarepta Therapeutics, Inc., (the "Registrant");

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the Registrant as of, and for, the periods presented in this report;

4. The Registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the Registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the Registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the Registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the Registrant's internal control over financial reporting that occurred during the Registrant's most recent fiscal quarter (the Registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the Registrant's internal control over financial reporting; and

5. The Registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Registrant's auditors and the audit committee of the Registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the Registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the Registrant's internal control over financial reporting.

November 7, 2019

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

EXHIBIT 31.2

CERTIFICATION

I, Sandesh Mahatme, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Sarepta Therapeutics, Inc., (the "Registrant");

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the Registrant as of, and for, the periods presented in this report;

4. The Registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the Registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the Registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) Evaluated the effectiveness of the Registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(d) Disclosed in this report any change in the Registrant's internal control over financial reporting that occurred during the Registrant's most recent fiscal quarter (the Registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the Registrant's internal control over financial reporting; and

5. The Registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the Registrant's auditors and the audit committee of the Registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the Registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the Registrant's internal control over financial reporting.

November 7, 2019

/s/ SANDESH MAHATME

Sandesh Mahatme
Executive Vice President,
Chief Financial Officer and
Chief Business Officer
(Principal Financial and Accounting Officer)

EXHIBIT 32.1

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Douglas S. Ingram, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended September 30, 2019, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

November 7, 2019

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

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.

EXHIBIT 32.2

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Sandesh Mahatme, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended September 30, 2019, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

November 7, 2019

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

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.