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

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended September 30, 2017

OR

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

Commission file number 001-14895

SAREPTA THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

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

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

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

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

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

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

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

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

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

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

SAREPTA THERAPEUTICS, INC.

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PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

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

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

Deferred rent and other	6,105	5,949
Total liabilities	103,125	87,413
Commitments and contingencies (Note 18)		
Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 99,000,000 shares authorized; 64,567,418 and 54,759,234 issued and outstanding at September 30, 2017 and December 31, 2016, respectively	6	5
Additional paid-in capital	1,890,172	1,503,126
Accumulated other comprehensive loss	(12)	(120)
Accumulated deficit	(1,193,010)	(1,166,320)
Total stockholders' equity	697,156	336,691
Total liabilities and stockholders' equity	$800,281	$424,104

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(unaudited, in thousands, except per share amounts)

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

See accompanying notes to unaudited condensed consolidated financial statements.

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SAREPTA THERAPEUTICS, INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

(unaudited, in thousands)

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

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

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(Unaudited)

1. BUSINESS

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

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

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

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

2. SIGNIFICANT ACCOUNTING POLICIES AND RECENT ACCOUNTING PRONOUNCEMENTS

Basis of Presentation

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

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

Estimates and Uncertainties

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

Concentration of Credit Risk

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

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

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

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

Significant Accounting Policies

For details about the Company's accounting policies, please read Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements of the Annual Report on Form 10-K for the year ended December 31, 2016.

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

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

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

Recent Accounting Pronouncements

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

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

In February 2016, the FASB issued ASU No. 2016-02, “Leases (Topic 842)”, which supersedes Topic 840, “Leases”. Under the new guidance, a lessee should recognize assets and liabilities that arise from its leases and disclose qualitative and quantitative information about its leasing arrangements. In transition, lessees and lessors are required to recognize and measure leases at the beginning of the earliest period presented using a modified retrospective approach. ASU No. 2016-02 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The adoption of this standard is expected to have an impact on the amount of the Company’s assets and liabilities. As of September 30, 2017, the Company has not elected to early adopt this guidance or determined the effect that the adoption of this guidance will have on its consolidated financial statements.

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

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

Reclassification

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

Subsequent Events

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

3. LITIGATION SETTLEMENT AND LICENSE AGREEMENTS

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

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

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

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

4. GAIN FROM SALE OF PRIORITY REVIEW VOUCHER

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

5. FAIR VALUE MEASUREMENTS

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

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

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

	Fair Value Measurement as of September 30, 2017			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Money market funds	$360,612	$360,612	$—	$ —
Commercial paper	83,506	—	83,506	—
Government and government agency bonds	113,934	—	113,934	—
Certificates of deposit	648	648	—	—
Total assets	$558,700	$361,260	$197,440	$ —

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

The Company's assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds and certificates of deposit. Money market funds are publicly traded mutual funds and are presented as cash equivalents in the unaudited condensed consolidated balance sheets as of September 30, 2017.

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

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

6. CASH, CASH EQUIVALENTS AND SHORT-TERM INVESTMENTS

It is the Company's policy to mitigate credit risk in its financial assets by maintaining a well-diversified portfolio that limits the amount of exposure as to maturity and investment type. There were no available-for-sale securities as of September 30, 2017. The weighted average maturity of the Company's available-for-sale securities as of December 31, 2016 was approximately four months.

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

	As of September 30, 2017			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$420,190	$ —	$ —	$420,190
Commercial paper	83,516	—	(10)	83,506
Government and government agency bonds	113,936	1	(3)	113,934
Total assets	$617,642	$ 1	$ (13)	$617,630
As reported:				
Cash and cash equivalents	$617,642	$ 1	$ (13)	$617,630
Total assets	$617,642	$ 1	$ (13)	$617,630

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

7. ACCOUNTS RECEIVABLE AND RESERVES FOR PRODUCT SALES

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

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

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

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

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

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

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

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

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

8. INVENTORY

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

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

9. ASSET HELD FOR SALE

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

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

10. OTHER CURRENT ASSETS AND OTHER NON-CURRENT ASSETS

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

	As of September 30, 2017	As of December 31, 2016
	(in thousands)	
Manufacturing-related deposits and prepaids	$18,399	$23,604
Prepaid clinical and preclinical expenses	3,612	1,225
Other prepaids	4,000	1,152
Other	1,022	914
Total other current assets	$27,033	$26,895

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

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

11. ACCRUED EXPENSES

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

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

12. INDEBTEDNESS

Term Loan

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

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

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

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

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

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

Revolving Line of Credit

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

Mortgage Loans

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

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

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

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

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

(1) Includes interest

13. EQUITY OFFERINGS

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

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

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

14. RESTRUCTURING

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

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

For the three months ended September 30, 2017, the restructuring expenses were de minimis. For the nine months ended September 30, 2017, the Company recorded $2.8 million of restructuring expenses, including $2.4 million related to the closure of Corvallis facility. For the three and nine months ended September 30, 2016, the Company recorded $1.3 million and $2.4 million of restructuring expenses, respectively, $1.0 million and $2.1 million, respectively, of which related to workforce reduction.

The following tables summarize the restructuring expenses by function for the periods indicated:

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

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

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

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

15. STOCK-BASED COMPENSATION

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

	For the Three Months Ended September 30,				For the Nine Months Ended September 30,			
	2017		2016		2017		2016	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options	221,398	$ 21.07	1,050	$ 37.38	4,678,357 (1)	$ 14.49	1,214,426	$ 11.96
Restricted stock units	—	$ —	—	$ —	181,029 (3)	$ 33.03	—	$ —
Restricted stock awards	—	$ —	91,778	$ 48.94	341,500 (2)	$ 34.58	117,553	$ 41.22

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

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

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

Stock-based Compensation Expense

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

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

The following table summarizes stock-based compensation expense by grant type included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2017	2016	2017	2016
	(in thousands)		(in thousands)	
Stock options	$5,420	$8,778	$17,221	$20,248
Restricted stock awards/units	954	232	4,408	689
Stock appreciation rights	—	115	—	345
Employee stock purchase plan	548	448	1,470	1,811
Total stock-based compensation expense	$6,922	$9,573	$23,099	$23,093

16. INCOME TAXES

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

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

17. NET LOSS PER SHARE

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

	For the Three Months Ended		For the Nine Months Ended	
	September 30,		September 30,	
	2017	2016	2017	2016
	(in thousands, except per share amounts)			
Net loss	$(47,734)	$(56,742)	$(26,690)	$(178,813)
Weighted-average number of shares of common stock and common stock equivalents outstanding:				
Weighted-average number of shares of common stock outstanding for computing basic loss per share	61,528	48,254	57,166	46,709
Dilutive effect of outstanding stock awards and stock options after application of the treasury stock method*	—	—	—	—
Weighted-average number of shares of common stock and dilutive common stock equivalents outstanding for computing diluted loss per share	61,528	48,254	57,166	46,709
Net loss per share - basic and diluted	$(0.78)	$(1.18)	$(0.47)	$(3.83)

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

18. COMMITMENTS AND CONTINGENCIES

Milestone Obligations

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

Other Funding Commitments

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

Litigation

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

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

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

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

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

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

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

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

This section should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and the section contained in our Annual Report on Form 10-K for the year ended December 31, 2016 under the caption "Part II-Item 7 — Management's Discussion and Analysis of Financial Condition and Results of Operations". This discussion contains certain forward-looking statements, which are often identified by words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may," "esti "could," "continue," "ongoing," "predict," "potential," "likely," "seek" and other similar expressions, as well as variations or negatives of these words. These statements contain projections of future results of operations or financial condition, or state other "forward-looking" information. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to:

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

other contracts;

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

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

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

Overview

U.S. Approval, MAA, and MAP

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

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

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

Our RNA-targeted Technologies

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

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

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

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

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

Our Clinical Programs

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

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

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

Manufacturing

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

Cash, Cash Equivalents and Investments

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

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

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

Critical Accounting Policies and Estimates

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

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

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

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

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

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

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

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

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

Revenues

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

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

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

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

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

Research and Development Expenses

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

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

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

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

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

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

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

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

	For the Nine Months Ended

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

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

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

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

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

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

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

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

Selling, General and Administrative Expenses

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

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

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

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

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

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

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

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

Settlement and License Charges

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

Amortization of In-licensed Rights

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

Gain from Sale of Priority Review Voucher

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

Interest income (expense) and other, net

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

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

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

Income tax expense

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

Liquidity and Capital Resources

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

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

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

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

- our ability to continue to generate revenues from sales of EXONDYS 51 and potential future products;
- the timing and costs associated with our global expansion;