BIOMARIN PHARMACEUTICAL INC
Form 10-Q
May 05, 2015

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-Q

(Mark One)

x QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended March 31, 2015

Or

o 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: 000-26727

BioMarin Pharmaceutical Inc.

(Exact name of registrant as specified in its charter)

Delaware	68-0397820
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

770 Lindaro Street, San Rafael, California 94901

(Address of principal executive offices) (Zip Code)

(415) 506-6700

(Registrant's telephone number including area code)

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 x No o

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 x No o

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.

Large accelerated filer x Accelerated filer o

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

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

Applicable only to issuers involved in bankruptcy proceedings during the preceding five years:

Indicate by check mark whether the registrant has filed all documents and reports required to be filed by Sections 12, 13 or 15(d) of the Securities Exchange Act of 1934 subsequent to the distribution of securities under a plan confirmed by a court. Yes o No o

Applicable only to corporate issuers:

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date: 160,336,762 shares of common stock, par value $0.001, outstanding as of April 24, 2015.

BIOMARIN PHARMACEUTICAL INC.

TABLE OF CONTENTS

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

March 31, 2015 and December 31, 2014

(In thousands of U.S. dollars, except per share amounts)

	March 31, 2015	December 31, 2014[1]
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$900,570	$875,486
Short-term investments	108,119	69,706
Accounts receivable, net (allowance for doubtful accounts: $489 and $490, at March 31, 2015 and December 31, 2014, respectively)	175,738	144,472
Inventory	222,833	199,452
Current deferred tax assets	31,203	31,203
Other current assets	84,265	111,835
Total current assets	1,522,728	1,432,154
Noncurrent assets:		
Investment in BioMarin/Genzyme LLC	890	1,039
Long-term investments	223,920	97,856
Property, plant and equipment, net	538,117	523,516
Intangible assets, net	926,896	156,578
Goodwill	202,392	54,258
Long-term deferred tax assets	163,411	159,771
Other assets	80,332	65,281
Total assets	$3,658,686	$2,490,453
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$230,212	$231,844
Short-term contingent acquisition consideration payable	75,294	3,895
Total current liabilities	305,506	235,739
Noncurrent liabilities:		
Long-term convertible debt	655,491	657,976
Long-term contingent acquisition consideration payable	39,052	38,767
Long-term deferred tax liabilities	193,202	—
Other long-term liabilities	39,980	30,077
Total liabilities	1,233,231	962,559
Stockholders' equity:		
Common stock, $0.001 par value: 250,000,000 shares authorized at March 31, 2015 and December 31, 2014: 160,282,313 and 149,093,647 shares issued and outstanding at March 31, 2015 and December 31, 2014, respectively	161	149
Additional paid-in capital	3,308,137	2,359,744

Company common stock held by Nonqualified Deferred Compensation Plan	(9,391)	(9,695)
Accumulated other comprehensive income	43,819	27,466
Accumulated deficit	(917,271)	(849,770)
Total stockholders' equity	2,425,455	1,527,894
Total liabilities and stockholders' equity	$3,658,686	$2,490,453

(1) December 31, 2014 balances were derived from the audited Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2014, filed with the Securities and Exchange Commission (the SEC) on March 2, 2015.

The accompanying notes are an integral part of these Condensed Consolidated Financial Statements.

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

Three Months Ended March 31, 2015 and 2014

(In thousands of U.S. dollars, except per share amounts)

(Unaudited)

	2015	2014
REVENUES:		
Net product revenues	$201,312	$149,004
Collaborative agreement revenues	376	415
Royalty, license and other revenues	1,576	2,133
Total revenues	203,264	151,552
OPERATING EXPENSES:		
Cost of sales	32,813	22,816
Research and development	142,074	86,166
Selling, general and administrative	92,806	60,069
Intangible asset amortization and contingent consideration	1,431	8,957
Total operating expenses	269,124	178,008
LOSS FROM OPERATIONS	(65,860)	(26,456)
Equity in the loss of BioMarin/Genzyme LLC	(150)	(338)
Interest income	683	1,123
Interest expense	(9,462)	(9,106)
Debt conversion expense	(163)	—
Other income	249	153
LOSS BEFORE INCOME TAXES	(74,703)	(34,624)
Provision for (benefit from) income taxes	(7,202)	3,491
NET LOSS	$(67,501)	$(38,115)
NET LOSS PER SHARE, BASIC	$(0.43)	$(0.26)
NET LOSS PER SHARE, DILUTED	$(0.43)	$(0.27)
Weighted average common shares outstanding, basic	157,612	143,983
Weighted average common shares outstanding, diluted	157,612	144,157
COMPREHENSIVE LOSS	$(51,148)	$(35,858)

The accompanying notes are an integral part of these Condensed Consolidated Financial Statements.

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

Three Months Ended March 31, 2015 and 2014

(In thousands of U.S. dollars)

(Unaudited)

	2015	2014
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$(67,501)	$(38,115)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	14,397	12,004
Non-cash interest expense	7,000	6,698
Accretion of discount on investments	417	1,900
Stock-based compensation	23,714	17,267
Gain on termination of lease	—	(8,858)
Equity in the loss of BioMarin/Genzyme LLC	150	338
Deferred income taxes	(7,800)	(179)
Excess tax benefit from stock option exercises	(527)	(278)
Unrealized foreign exchange gain on forward contracts	(5,686)	1,323
Non-cash changes in the fair value of contingent acquisition consideration payable	282	8,151
Debt conversion expense	163	—
Other	(443)	—
Changes in operating assets and liabilities:		
Accounts receivable, net	(26,789)	7,406
Inventory	(23,946)	(13,870)
Other current assets	(3,522)	(927)
Other assets	330	(920)
Accounts payable and accrued liabilities	(57,236)	(19,020)
Other long-term liabilities	9,186	587
Net cash used in operating activities	(137,811)	(26,493)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property, plant and equipment	(43,832)	(23,607)
Maturities and sales of investments	124,137	69,391
Purchase of available-for-sale investments	(288,431)	(84,306)
Purchase of promissory note	(3,326)	—
Business acquisitions, net of cash acquired	(538,392)	—
Other	(1,027)	—
Net cash used in investing activities	(750,871)	(38,522)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from exercises of stock options and Employee Stock Purchase Plan (the ESPP)	28,026	19,712
Taxes paid related to net share settlement of equity awards	(735)	(473)
Proceeds from public offering of common stock, net	888,257	117,464
Excess tax benefit from stock option exercises	527	278
Payments for debt conversion	(163)	—
Other	(1,121)	(17)

Net cash provided by financing activities	914,791	136,964
Effect of exchange rate changes on cash	(1,025)	(952)
NET INCREASE IN CASH AND CASH EQUIVALENTS	25,084	70,997
Cash and cash equivalents:		
Beginning of period	$875,486	$568,781
End of period	$900,570	$639,778
SUPPLEMENTAL CASH FLOW DISCLOSURES:		
Cash paid for interest, net of interest capitalized into fixed assets	309	1
Cash paid for income taxes	1,358	381
Stock-based compensation capitalized into inventory	2,480	2,053
Depreciation capitalized into inventory	3,580	2,924
SUPPLEMENTAL CASH FLOW DISCLOSURES FROM INVESTING AND FINANCING ACTIVITIES:		
Decrease in accounts payable and accrued liabilities related to fixed assets	(20,985)	(9,171)
Conversion of convertible debt	8,133	—
Deferred offering costs reclassified into additional paid-in-capital as a result of conversion of convertible debt	45	—
Release of escrow balance for purchase of San Rafael Corporate Center	—	116,500

The accompanying notes are an integral part of these Condensed Consolidated Financial Statements.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(1) NATURE OF OPERATIONS AND BUSINESS RISKS

BioMarin Pharmaceutical Inc. (the Company or BioMarin), a Delaware corporation, develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. BioMarin selects product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products. The Company's product portfolio is comprised of five approved products and multiple clinical and pre-clinical product candidates. The Company's approved products are Vimizim (elosulfase alpha), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

Through March 31, 2015, the Company had accumulated losses of approximately $917.3 million. The Company expects to continue to finance future cash needs that exceed its operating activities primarily through its current cash, cash equivalents, short-term and long-term investments, and to the extent necessary, through proceeds from equity or debt financings, loans and collaborative agreements with corporate partners. If the Company elects to increase its spending on development programs significantly above current long-term plans or enters into potential licenses and other acquisitions of complementary technologies, products or companies, the Company may need additional capital.

The Company is subject to a number of risks, including: the financial performance of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse; the potential need for additional financings; the Company's ability to successfully commercialize its approved product candidates; the uncertainty of the Company's research and development (R&D) efforts resulting in future successful commercial products; the Company's ability to successfully obtain regulatory approval for new products; significant competition from larger organizations; reliance on the proprietary technology of others; dependence on key personnel; uncertain patent protection; dependence on corporate partners and collaborators; and possible restrictions on reimbursement from governmental agencies and healthcare organizations, as well as other changes in the health care industry.

(2) BASIS OF PRESENTATION

The accompanying Condensed Consolidated Financial Statements have been prepared pursuant to the rules and regulations of the SEC for Quarterly Reports on Form 10-Q and do not include all of the information and note disclosures required by U.S. generally accepted accounting principles (U.S. GAAP) for complete financial statements. The Condensed Consolidated Financial Statements should therefore be read in conjunction with the Consolidated Financial Statements and Notes thereto for the fiscal year ended December 31, 2014 included in the Company's Annual Report on Form 10-K.

The accompanying Condensed Consolidated Financial Statements have been prepared in accordance with U.S. GAAP, which requires management to make estimates and assumptions that affect amounts reported in the Condensed Consolidated Financial Statements and accompanying disclosures. Although these estimates are based on management's best knowledge of current events and actions that the Company may undertake in the future, actual results may be different from those estimates. The Condensed Consolidated Financial Statements reflect all adjustments of a normal, recurring nature that are, in the opinion of management, necessary for a fair presentation of

results for these interim periods. The results of operations for the three months ended March 31, 2015 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2015.

The Company has evaluated events and transactions subsequent to the balance sheet date. Based on this evaluation, the Company is not aware of any events or transactions that occurred subsequent to the balance sheet date but prior to filing this Quarterly Report on Form 10-Q that would require recognition or disclosure in the Condensed Consolidated Financial Statements.

(3) SIGNIFICANT ACCOUNTING POLICIES

There have been no material changes to the Company's significant accounting policies during the three months ended March 31, 2015, as compared to the significant accounting policies disclosed in Note 3 of the Consolidated Financial Statements in the Company's Annual Report on Form 10-K for the year ended December 31, 2014.

Reclassifications

Certain items in the Company's prior year Condensed Consolidated Financial Statements have been reclassified to conform to the current presentation.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(4) RECENT ACCOUNTING PRONOUNCEMENTS

There have been no new accounting pronouncements or changes to accounting pronouncements during the three months ended March 31, 2015, as compared to the recent accounting pronouncements described in Note 4 of the Company's Annual Report on Form 10-K for the year-ended December 31, 2014, that are of significance or potential significance to the Company.

(5) ACQUISITIONS

Prosensa Holding N.V.

On January 29 2015, the Company completed the acquisition of Prosensa Holding N.V. (Prosensa), a public limited liability company organized under the laws of the Netherlands, for a total purchase price of $751.5 million. In connection with the acquisition of Prosensa, the Company recognized transaction costs of $9.7 million, of which $2.7 million and $7.0 million, respectively, was recognized in the year ended December 31, 2014 and the three months ended March 31, 2015.

Prosensa was an innovative biotechnology company engaged in the discovery and development of ribonucleic acid (RNA)-modulating therapeutics for the treatment of genetic disorders. Prosensa's primary focus was on rare neuromuscular and neurodegenerative disorders with a large unmet medical need, including subsets of patients with Duchenne muscular dystrophy (DMD), myotonic dystrophy and Huntington's disease. Prosensa's clinical portfolio of RNA-based product candidates was focused on the treatment of DMD. Each of Prosensa's DMD compounds has been granted orphan drug status in the United States (the U.S.) and the European Union (the EU). Prosensa's lead product, drisapersen, is currently under a rolling review as part of a rolling new drug application (NDA) with the Food and Drug Administration (the FDA). On April 27, 2015, the Company announced the completion of the rolling submission of the NDA to the FDA. The Company expects to file a marketing authorization application (MAA) for drisapersen with the European Medicines Agency (the EMA) in the summer of 2015.

In connection with its acquisition of Prosensa, the Company made cash payments totaling $680.1 million which were comprised of $620.7 million for approximately 96.8% of Prosensa's ordinary shares (the Prosensa Shares), $38.6 million for the options that vested pursuant to the Company's tender offer for the Prosensa Shares and $20.8 million to the remaining Prosensa shareholders that did not tender their shares under the tender offer. Additionally, for each Prosensa Share, the Company issued one non-transferable contingent value right (the CVR), which represents the contractual right to receive a cash payment of up to $4.14 per Prosensa Share, or approximately $160.0 million (undiscounted), upon the achievement of certain product approval milestones. The fair value of the CVRs and acquired in-process research and development (IPR&D) on the acquisition date was $71.4 million and $772.8 million, respectively. The acquisition date fair value of the CVRs and IPR&D was estimated by applying a probability-based income approach utilizing an appropriate discount rate. Key assumptions include a discount rate and various probability factors. See Note 15 to these Condensed Consolidated Financial Statements for additional discussion regarding fair value measurements of the CVRs which is included in contingent acquisition consideration payable.

7

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The following table presents the allocation of the purchase consideration for the Prosensa acquisition, including the CVRs, based on fair value.

Cash and cash equivalents	$141,669
Trade accounts receivable	3,086
Other current assets	1,537
Property, plant and equipment	2,683
Intangible assets	497
Other assets	104
Acquired IPR&D	772,808
Total identifiable assets acquired	922,384
Accounts payable and accrued expenses	(68,799)
Debt assumed	(57,053)
Deferred tax liability	(193,202)
Total liabilities assumed	(319,054)
Net identifiable assets acquired	603,330
Goodwill	148,134
Net assets acquired	$751,464

A substantial portion of the assets acquired consisted of IPR&D related to Prosensa's product candidates drisapersen and exons PRO 044 and PRO 045, which are considered to be indefinite-lived assets until completion or abandonment of the associated research and development (R&D) efforts. The Company determined that the estimated acquisition-date fair value of the intangible assets related to drisapersen and Prosensa's other primary product candidates, PRO 044 and PRO-045 was $731.8 million, $16.9 million and $24.1 million, respectively.

The deferred tax liability relates to the tax impact of future amortization or possible impairments associated with the identified intangible assets acquired, which are not deductible for tax purposes.

Prosensa's results of operations prior to and since the acquisition date are insignificant to the Company's Condensed Consolidated Financial Statements.

See Note 10 to these Condensed Consolidated Financial Statements for further discussion of the indefinite-lived intangible assets.

San Rafael Corporate Center

In March 2014, the Company completed the acquisition of the real estate commonly known as the San Rafael Corporate Center (SRCC), located in San Rafael, California. SRCC is a multi-building, commercial property where, prior to the acquisition, the Company was leasing a certain portion of the space for its headquarters and related operating activities. The purpose of this acquisition is to allow for future expansion of the Company's corporate headquarters to accommodate anticipated headcount growth. The acquisition of SRCC has been accounted for as a business combination because the building and the in-place leases met the definition of a business in Accounting Standards Codification 805 (ASC 805), Business Combinations. The fair value of the consideration paid for SRCC was $116.5 million, all of which was paid in cash, which was held in escrow as of December 31, 2013.

8

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The following table summarizes the estimated fair values of assets acquired as of the date of acquisition:

	Estimated Fair Value	Estimated Useful Lives
Building and improvements	$ 94,414	50 years
Land	14,565	
Land improvements	3,616	10 years
Intangible assets	3,905	Remaining lease terms
Total identifiable net assets	$ 116,500	

The fair values assigned to tangible and identifiable intangible assets acquired are based on management's estimates and assumptions using the information that was available as of the date of the acquisition. The Company believes that the information provides a reasonable basis for estimating the fair values of assets acquired.

The following table sets forth the fair value of the components of the identifiable intangible assets acquired by asset class as of the date of acquisition:

Above market leases	$351
In-place leases	3,554
Total intangible assets subject to amortization	$3,905

The value of any in-place leases is estimated to be equal to the property owners' avoidance of costs necessary to release the property for a lease term equal to the remaining primary in-place lease term and the value of investment-grade tenancy, which is derived by estimating, based on a review of the market, the cost to be borne by a property owner to replicate a market lease for the remaining in-place term. These costs consist of: (i) rent lost during downtime (e.g., assumed periods of vacancy), (ii) estimated expenses that would be incurred by the property owner during periods of vacancy, (iii) rent concessions (e.g., free rent), (iv) leasing commissions and (v) tenant improvement allowances. The Company determined these values using management's estimates along with third-party appraisals. The Company will amortize the capitalized value of in-place lease intangible assets to expense over the remaining initial term of each lease. The Company will amortize the capitalized value of above market leases to expense over the remaining lives of the underlying leases.

The amount of third-party tenant revenue (included in the line item Royalty, License and Other Revenues) included in the Company's Consolidated Statements of Comprehensive Loss for the three months ended March 31, 2015, was $1.0 million, compared to $0.4 million for the three months ended March 31, 2014. The amount of net income/loss from third-party tenants for the three months ended March 31, 2015 and 2014, was insignificant to the Company's Consolidated Statement of Comprehensive Loss.

SRCC's results of operations prior to the acquisition were insignificant to the Company's Condensed Consolidated Financial Statements.

Included in Selling, General and Administrative (SG&A) expenses during the three months ended March 31, 2014 are transaction costs incurred in connection with the acquisition of SRCC of $0.2 million. The Company recognized a gain of $8.8 million in the three months ended March 31, 2014, due to the early termination of the Company's pre-existing lease and the realization of the remaining balance in deferred rent and the reversal of the related asset retirement obligation upon acquisition of the SRCC. $2.7 million and $6.1 million of the gain were included in SG&A and R&D expenses, respectively, which is consistent with the Company's allocation practices for facility costs for this previously leased space.

(6) STOCKHOLDERS' EQUITY

In January 2015, the Company sold 9,775,000 shares of its common stock at a price of $93.25 per share in an underwritten public offering pursuant to an effective registration statement previously filed with the SEC. The Company received net proceeds of approximately $888.3 million from this public offering after underwriter's discount and offering costs.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(7) NET LOSS PER COMMON SHARE

Potentially issuable shares of common stock include shares issuable upon the exercise of outstanding employee stock option awards, common stock issuable under the Company's Amended and Restated 2006 ESPP, unvested restricted stock, common stock held by the Company's Nonqualified Deferred Compensation Plan (the NQDC) and contingent issuances of common stock related to convertible debt.

The following table sets forth the computation of basic and diluted earnings per common share (in thousands of common shares):

	Three Months Ended March 31,	
	2015	2014
Numerator:		
Net loss, basic	$(67,501)	$(38,115)
Gain on Company common stock issued to the NQDC	—	(374)
Net loss, diluted	$(67,501)	$(38,489)
Denominator:		
Weighted-average common shares outstanding, basic	157,612	143,983
Effect of dilutive securities:		
Common stock issued to the NQDC	—	174
Weighted-average common shares outstanding, diluted	157,612	144,157
Net loss per common share, basic	$(0.43)	$(0.26)
Net loss per common share, diluted	$(0.43)	$(0.27)

In addition to the equity instruments included in the table above, the table below presents potential shares of common stock that were excluded from the computation as they were anti-dilutive using the treasury stock method (in thousands of common shares):

	Three Months Ended March 31,	
	2015	2014
Options to purchase common stock	11,109	12,444
Common stock issuable under the 2017 Notes	1,567	3,047
Common stock issuable under the 2018 and 2020 Notes	7,966	7,966
Unvested restricted stock units	1,557	1,326
Potentially issuable common stock for ESPP purchases	223	209
Common stock held by the NQDC	213	—

Total number of potentially issuable shares	22,635	24,992

The effect of the Company's 0.75% senior subordinated convertible notes due in 2018 (the 2018 Notes) and the Company's 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes and together with the 2018 Notes, the Notes) was excluded from the diluted net loss per common share since they may be settled in cash or shares at the Company's option and the Company's current intention is to settle up to the principal amount of the converted notes in cash and any excess conversion value (conversion spread) in shares of the Company's common stock. As a result, during the three months ended March 31, 2014 the 2018 Notes and the 2020 Notes had no effect on diluted net loss per share until the Company's stock price exceeded the conversion price of $94.15 per share for the Notes. Although the Company's stock price exceeded the conversion price at March 31, 2105, the potential shares issuable under the Notes were excluded from the calculation of diluted loss per share as they were anti-dilutive using the if-converted method.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(8) INVESTMENTS

All investments were classified as available-for-sale at March 31, 2015 and December 31, 2014. The amortized cost, gross unrealized holding gains or losses, and fair value of the Company's available-for-sale securities by major security type at March 31, 2015 and December 31, 2014 are summarized in the tables below:

	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value at March 31, 2015
Certificates of deposit	$ 69,871	$ 1	$ —	$ 69,872
Corporate debt securities	147,537	129	—	147,666
Commercial paper	21,354	—	—	21,354
U.S. government agency securities	93,012	31	—	93,043
Greek government-issued bonds	50	54	—	104
Total	$ 331,824	$ 215	$ —	$ 332,039

	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value at December 31, 2014
Certificates of deposit	$ 72,302	$ 1	$ —	$ 72,303
Corporate debt securities	95,478	—	(342)	95,136
Greek government-issued bonds	50	73	—	123
Total	$ 167,830	$ 74	$ (342)	$ 167,562

The Company has two investments in marketable equity securities measured using quoted prices in their respective active markets and certain interest in non-marketable equity securities that are collectively considered strategic investments. As of March 31, 2015, the fair value of the Company's marketable equity securities was $41.8 million, which included an unrealized gain of $29.3 million. The carrying cost of the non-marketable securities was $3.1 million at March 31, 2015. As of December 31, 2014, the fair value of the Company's marketable equity securities was $30.8 million, which included an unrealized gain of $18.3 million. These investments are recorded in Other Assets in the Company's Condensed Consolidated Balance Sheets.

The fair values of available-for-sale securities by contractual maturity were as follows:

	March 31, 2015	December 31, 2014
Maturing in one year or less	$108,119	$69,706
Maturing after one year through five years	223,920	97,856
Total	$332,039	$167,562

Impairment assessments are made at the individual security level each reporting period. When the fair value of an investment is less than its cost at the balance sheet date, a determination is made as to whether the impairment is other-than-temporary and, if it is other-than-temporary, an impairment loss is recognized in earnings equal to the difference between the investment's amortized cost and fair value at such date. As of March 31, 2015, some of the Company's investments were in an unrealized loss position. However, the Company has the ability and intent to hold all investments that have been in a continuous loss position until maturity or recovery, thus no other-than-temporary impairment is deemed to have occurred.

See Note 15 to these Condensed Consolidated Financial Statements for additional discussion regarding the fair value of the Company's available-for-sale securities.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(9) GOODWILL

Goodwill is tested for impairment on an annual basis and between annual tests if the Company becomes aware of any events occurring or changes in the circumstances that would indicate a reduction in the fair value of the goodwill below its carrying amount.

The following table represents the changes in goodwill for the three months ended March 31, 2015:

Balance at December 31, 2014	$54,258
Addition of goodwill related to the acquisition of Prosensa	148,134
Balance at March 31, 2015	$202,392

(10) INTANGIBLE ASSETS

Intangible assets consisted of the following:

	March 31, 2015	December 31, 2014
Intangible assets:		
Finite-lived intangible assets	$123,836	$123,365
Indefinite-lived intangible assets	847,238	74,430
Gross intangible assets:	971,074	197,795
Less: Accumulated amortization	(44,178)	(41,217)
Net carrying value	$926,896	$156,578

Indefinite-Lived Intangible Assets

IPR&D assets are considered to be indefinite-lived until the completion or abandonment of the associated R&D efforts. During the period the assets are considered indefinite-lived, they will not be amortized but will be tested for impairment on an annual basis and between annual tests if the Company becomes aware of any events occurring or changes in circumstances that would indicate a reduction in the fair value of the IPR&D assets below their respective carrying amounts. If and when development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets would be deemed finite-lived and would then be amortized based on their respective estimated useful lives at that point in time.

See Note 6 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2014 for additional information related to the Company's Intangible Assets.

(11) PROPERTY, PLANT AND EQUIPMENT

Property, plant and equipment, net consisted of the following:

	March 31, 2015	December 31, 2014
Leasehold improvements	$40,733	$39,297
Building and improvements	339,161	335,991
Manufacturing and laboratory equipment	129,348	124,564
Computer hardware and software	99,879	97,032
Furniture and equipment	14,874	13,717
Land improvements	4,106	4,106
Land	29,357	29,358
Construction-in-progress	120,971	108,340
	778,429	752,405
Less: Accumulated depreciation	(240,312)	(228,889)
Total property, plant and equipment, net	$538,117	$523,516

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

Depreciation expense for the three months ended March 31, 2015 and 2014 was $11.5 million and $9.6 million, respectively, of which $3.6 million and $2.9 million, respectively, was capitalized into inventory.

Capitalized interest related to the Company's property, plant and equipment purchases for each of the three months ended March 31, 2015 and 2014 was insignificant.

(12) SUPPLEMENTAL BALANCE SHEET INFORMATION

Inventory consisted of the following:

	March 31, 2015	December 31, 2014
Raw materials	$21,690	$22,488
Work-in-process	124,192	114,393
Finished goods	76,951	62,571
Total inventory	$222,833	$199,452

Other Current Assets consisted of the following:

	March 31, 2015	December 31, 2014
Prepaid expenses	39,159	35,390
Short-term forward currency exchange contract assets	18,337	10,513
Promissory notes receivable, net	3,326	46,946
Restricted investments	7,131	2,354
Convertible promissory note conversion option	—	2,386
Other receivables	10,983	9,733
Other	5,329	4,513
Total other current assets	$84,265	$111,835

Other Assets consisted of the following:

	March 31, 2015	December 31, 2014
Deposits	11,045	12,021
Deferred debt offering costs	10,911	11,763
Strategic investments	44,918	30,811
Long-term forward foreign currency exchange contract assets	7,391	5,387
Other	6,067	5,299
Total other assets	$80,332	$ 65,281

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

Accounts payable and accrued liabilities consisted of the following:

	March 31, 2015	December 31, 2014
Accounts payable	$23,606	$32,779
Accrued accounts payable	106,462	98,490
Accrued compensation expense	32,461	45,479
Accrued vacation expense	15,871	12,540
Accrued rebates payable	17,173	14,859
Accrued royalties payable	7,207	9,050
Value added taxes payable	5,730	5,479
Other accrued operating expenses	8,721	8,244
Other	12,981	4,924
Total accounts payable and accrued liabilities	$230,212	$231,844

(13) CONVERTIBLE DEBT

The following table summarizes information regarding the Company’s convertible debt:

	March 31, 2015	December 31, 2014
Convertible Notes due 2020, net of unamortized discount of $74,214 and $77,045, at March 31, 2015 and December 31, 2014, respectively	$300,786	$297,955
Convertible Notes due 2018, net of unamortized discount of $52,193 and $55,537, at March 31, 2015 and December 31, 2014, respectively	322,807	319,463
Convertible Notes due 2017	31,898	40,558
Total convertible debt, net of unamortized discount	$655,491	$657,976
Fair value of fixed rate convertible debt		

Convertible Notes due in 2020 [1]	$568,444	$456,360
Convertible Notes due in 2018 [1]	555,994	442,448
Convertible Notes due in 2017 [1]	195,370	180,984
Total	$1,319,808	$1,079,792

(1) The fair value of the Company's fixed rate convertible debt is based on open market trades and is classified as Level 1 in the fair value hierarchy.

Interest expense on the Company's convertible debt was comprised of the following:

	Three Months Ended March 31,	
	2015	2014
Coupon interest	$2,462	$2,408
Amortization of issuance costs	826	843
Accretion of debt discount	6,174	5,855
Total interest expense on convertible debt	$9,462	$9,106

During the three months ended March 31, 2015, the Company entered into separate agreements with three existing holders of its senior subordinated convertible notes due in 2017 (the 2017 Notes) pursuant to which such holders converted $8.1 million in aggregate principal amount of the 2017 Notes into 399,469 shares of the Company's common stock. In addition to issuing the requisite number of shares of the Company's common stock, the Company also made varying cash payments to the holder totaling

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

$0.2 million in aggregate, of which $0.2 million was recognized in total as Debt Conversion Expense on the Condensed Consolidated Statement of Comprehensive Loss for the three months ended March 31, 2015.

See Note 13 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2014 for additional information related to the Company's Convertible Debt.

(14) DERIVATIVE INSTRUMENTS AND HEDGING STRATEGIES

The Company uses forward foreign currency exchange contracts to hedge certain operational exposures resulting from potential changes in foreign currency exchange rates. Such exposures result from portions of the Company's forecasted revenues and operating expenses being denominated in currencies other than the U.S. dollar, primarily the Euro, the British Pound and the Brazilian Real.

The Company designates certain of these forward foreign currency exchange contracts as hedging instruments and enters into some forward foreign currency exchange contracts that are considered to be economic hedges that are not designated as hedging instruments. Whether designated or undesignated, these forward foreign currency exchange contracts protect against the reduction in value of forecasted foreign currency cash flows resulting from product revenues, royalty revenues, operating expenses and asset or liability positions designated in currencies other than the U.S. dollar. The fair values of forward foreign currency exchange contracts are estimated using current exchange rates and interest rates, and take into consideration the current creditworthiness of the counterparties or the Company, as applicable. Details of the specific instruments used by the Company to hedge its exposure to foreign currency exchange rate fluctuations are discussed below. See Note 15 to these Condensed Consolidated Financial Statements for additional discussion regarding the fair value of forward foreign currency exchange contracts.

At March 31, 2015, the Company had 94 forward foreign currency exchange contracts outstanding to sell a total of 131.6 million Euros and seven forward foreign currency exchange contracts outstanding to purchase 20.0 million Euros with expiration dates ranging from April 2015 through March 2018. These hedges were entered into in order to protect against the fluctuations in revenue associated with Euro-denominated product sales and operating expenses. The Company has formally designated these forward foreign currency exchange contracts as cash flow hedges and expects them to be highly effective in offsetting fluctuations in revenues denominated in Euros related to changes in foreign currency exchange rates.

The Company also enters into forward foreign currency exchange contracts that are not designated as hedges for accounting purposes. The changes in fair value of these forward foreign currency exchange contracts are included as a part of SG&A expense in the Company's Condensed Consolidated Statements of Comprehensive Loss. At March 31, 2015, the Company had one outstanding forward foreign currency exchange contract to sell 42.1 million Euros and one outstanding forward foreign currency exchange contract to sell 6.4 million British Pounds, both of which were not designated as a hedge for accounting purposes and matured on April 30, 2015.

The maximum length of time over which the Company is hedging its exposure to the reduction in value of forecasted foreign currency revenues through forward foreign currency exchange contracts is through March 2018. Over the next twelve months, the Company expects to reclassify $18.9 million from accumulated other comprehensive income to earnings as the forecasted revenue transactions occur.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

The fair value carrying amounts of the Company's derivative instruments were as follows:

	Asset Derivatives March 31, 2015 Balance Sheet Location	Fair Value	Liability Derivatives March 31, 2015 Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	$ 18,171	Accounts payable and accrued liabilities	$ —
Forward foreign currency exchange contracts	Other assets	7,391	Other long- term liabilities	—
Total		$ 25,562		$ —
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	$ 166	Other long- term liabilities	$ 805
Total		166		805
Total value of derivative contracts		$ 25,728		$ 805

	Asset Derivatives December 31, 2014 Balance Sheet Location	Fair Value	Liability Derivatives December 31, 2014 Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	$ 10,206	Accounts payable and accrued liabilities	$ —
Forward foreign currency exchange contracts	Other assets	5,387	Other long- term liabilities	—
Total		$ 15,593		$ —
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	$ 307	Accounts payable and accrued liabilities	$ 12
Total		307		12
Total value of derivative contracts		$ 15,900		$ 12

The effect of the Company's derivative instruments on the Condensed Consolidated Financial Statements for the three months ended March 31, 2015 and 2014 was as follows:

	Forward Foreign Currency Exchange Contracts Three Months Ended March 31,	
	2015	2014
Derivatives Designated as Hedging Instruments:		
Net gain recognized in Other Comprehensive Income (OCI) (1)	$ 13,776	$ 1,396
Net gain reclassified from accumulated OCI into income (2)	4,739	(567)
Net gain (loss) recognized in net loss (3)	141	(121)
Derivatives Not Designated as Hedging Instruments:		
Net gain recognized in net loss(4)	$ 7,800	$ 56

(1) Net change in the fair value of the effective portion classified as OCI.
(2) Effective portion classified as net product revenue.
(3) Ineffective portion and amount excluded from effectiveness testing classified as SG&A expense.
(4) Classified as SG&A expense.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

At March 31, 2015 and December 31, 2014, accumulated other comprehensive income before taxes associated with forward foreign currency exchange contracts qualifying for hedge accounting treatment was a gain of $24.5 million and a gain of $15.9 million, respectively.

The Company is exposed to counterparty credit risk on all of its derivative financial instruments. The Company has established and maintains strict counterparty credit guidelines and enters into hedges only with financial institutions that are investment grade or better to minimize the Company's exposure to potential defaults. The Company does not require collateral to be pledged under these agreements.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(15) FAIR VALUE MEASUREMENTS

The Company measures certain financial assets and liabilities at fair value on a recurring basis, including available-for-sale fixed income securities and foreign currency derivatives. The tables below present the fair value of these financial assets and liabilities determined using the following input levels.

	Fair Value Measurements at March 31, 2015			
	Quoted Price in Active Markets For Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash and cash equivalents:				
Overnight deposits	$833,545	$ —	$ —	$833,545
Money market instruments	—	67,025	—	67,025
Total cash and cash equivalents	833,545	67,025	—	900,570
Available-for-sale securities:				
Short-term:				
Certificates of deposit	—	57,176	—	57,176
Corporate debt securities	—	15,546	—	15,546
Commercial paper	—	21,354	—	21,354
U.S. government agency securities	—	14,043	—	14,043
Long-term:				
Certificates of deposit	—	12,696	—	12,696
Corporate debt securities	—	132,120	—	132,120
U.S. government agency securities	—	79,000	—	79,000
Greek government-issued bonds	—	104	—	104
Total available-for-sale securities	—	332,039	—	332,039
Other Current Assets:				
Nonqualified Deferred Compensation Plan assets	—	353	—	353
Forward foreign currency exchange contract[1]	—	18,337	—	18,337
Restricted investments [2]	—	7,131	—	7,131
Total other current assets	—	25,821	—	25,821
Other Assets:				

Nonqualified Deferred Compensation Plan assets	—	5,884	—	5,884
Forward foreign currency exchange contract[1]	—	7,391	—	7,391
Strategic investment [4]	41,809	—	—	41,809
Total other assets	41,809	13,275	—	55,084
Total assets	$875,354	$ 438,160	$ —	$1,313,514
Liabilities:				
Current Liabilities:				
Nonqualified Deferred Compensation Plan liability	$1,198	$ 353	$ —	$1,551
Contingent acquisition consideration payable	—	—	75,294	75,294
Total current liabilities	1,198	353	75,294	76,845
Other long-term liabilities:				
Nonqualified Deferred Compensation Plan liability	25,402	5,884	—	31,286
Forward foreign currency exchange contract [1]	—	805	—	805
Contingent acquisition consideration payable	—	—	39,052	39,052
Total other long-term liabilities	25,402	6,689	39,052	71,143
Total liabilities	$26,600	$ 7,042	$ 114,346	$147,988

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

	Fair Value Measurements at December 31, 2014 Quoted Price in Active Markets For Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash and cash equivalents:				
Overnight deposits	$225,159	$ —	$ —	$225,159
Money market instruments	—	650,327	—	650,327
Total cash and cash equivalents	225,159	650,327	—	875,486
Available-for-sale securities:				
Short-term:				
Certificates of deposit	—	54,174	—	54,174
Corporate debt securities	—	15,532	—	15,532
Long-term:				
Certificates of deposit	—	18,129	—	18,129
Corporate debt securities	—	79,604	—	79,604
Greek government-issued bonds	—	123	—	123
Total available-for-sale securities	—	167,562	—	167,562
Other Current Assets:				
Nonqualified Deferred Compensation Plan assets	—	514	—	514
Forward foreign currency exchange contract[1]	—	10,513	—	10,513
Restricted investments [2]	—	2,354	—	2,354
Embedded derivative [3]	—	—	2,386	2,386
Total other current assets	—	13,381	2,386	15,767
Other Assets:				
Nonqualified Deferred Compensation Plan assets	—	5,112	—	5,112
Restricted investments [2]	—	5,387	—	5,387
Strategic investment [4]	30,811	—	—	30,811
Total other assets	30,811	10,499	—	41,310
Total assets	$255,970	$ 841,769	$ 2,386	$1,100,125
Liabilities:				
Current Liabilities:				
Nonqualified Deferred Compensation Plan liability	$1,790	$ 514	$ —	$2,304
Forward foreign currency exchange contract[1]	—	12	—	12
Contingent acquisition consideration payable	—	—	3,895	3,895

Total current liabilities	1,790	526	3,895	6,211
Other long-term liabilities:				
Nonqualified Deferred Compensation Plan liability	18,453	5,112	—	23,565
Contingent acquisition consideration payable	—	—	38,767	38,767
Total other long-term liabilities	18,453	5,112	38,767	62,332
Total liabilities	$20,243	$ 5,638	$ 42,662	$68,543

(1) See Note 14 to these Condensed Consolidated Financial Statements for further information regarding the derivative instruments.

(2) The restricted investments at March 31, 2015 and December 31, 2014 secure the Company's irrevocable standby letter of credit obtained in connection with certain commercial agreements.

(3) The embedded derivative at December 31, 2014 represents the fair value of the conversion feature of a promissory note which may be settled in the issuer's underlying shares.

(4) The Company has investments in marketable equity securities measured using quoted prices in an active market that are considered strategic investments. See Note 8 to these Condensed Consolidated Financial Statements for additional discussion regarding the Company's strategic investments.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

There were no transfers between levels during the three months ended March 31, 2015.

The Company's Level 2 securities are valued using third-party pricing sources. The pricing services utilize industry standard valuation models, including both income and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include reported trades of and broker/dealer quotes on the same or similar securities, issuer credit spreads, benchmark securities, prepayment/default projections based on historical data and other observable inputs.

The Company validates the prices provided by its third-party pricing services by understanding the models used, obtaining market values from other pricing sources, analyzing pricing data in certain instances and confirming those securities traded in active markets. See Note 8 to these Condensed Consolidated Financial Statements for further information regarding the Company's financial instruments.

Liabilities measured at fair value using Level 3 inputs were comprised of contingent acquisition consideration payable and asset retirement obligations.

The Company's contingent acquisition consideration payable is estimated using a probability-based income approach utilizing an appropriate discount rate. Key assumptions used by management to estimate the fair value of contingent acquisition consideration payable include estimated probabilities, the estimated timing of when a milestone may be attained and assumed discount periods and rates. Subsequent changes in the fair value of the contingent acquisition consideration payable, resulting from management's revision of key assumptions, will be recorded in Intangible Asset Amortization and Contingent Consideration in the Company's Condensed Consolidated Statements of Comprehensive Loss. The probability-based income approach used by management to estimate the fair value of the contingent acquisition consideration is most sensitive to changes in the estimated probabilities.

Contingent acquisition consideration payable at December 31, 2014	$42,662
Addition of contingent consideration payable related to the Prosensa acquisition (CVR)	71,402
Changes in the fair value of the contingent acquisition consideration payable	282
Contingent acquisition consideration payable at March 31, 2015	$114,346

Under certain of the Company's lease agreements, the Company is contractually obligated to return leased space to its original condition upon termination of the lease agreement. The Company records an asset retirement obligation liability and a corresponding capital asset in an amount equal to the estimated fair value of the obligation when estimable. In subsequent periods, for each such lease, the Company records Interest Expense to accrete the asset retirement obligation liability to full value and depreciates each capitalized asset retirement obligation asset, both over the term of the associated lease agreement.

Asset retirement obligations at December 31, 2014	$3,765
Accretion expense	36
Additions	84
Asset retirement obligations at March 31, 2015	$3,885

The Company acquired intangible assets as a result of various business acquisitions. The estimated fair value of these long-lived assets was measured using Level 3 inputs as of the acquisition date.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(16) STOCK-BASED COMPENSATION

The Company's stock-based compensation plans include the Amended and Restated 2006 Share Incentive Plan (the Share Incentive Plan), the ESPP, the 2014 Inducement Plan and the 2012 Inducement Plan. The 2012 Inducement Plan expired in May 2013. The Company's stock-based compensation plans are administered by the Compensation Committee of the Board of Directors, which selects persons to receive awards and determines the number of shares subject to each award and the terms, conditions, performance measures and other provisions of the award. See Note 16 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2014, for additional information related to these stock-based compensation plans.

Determining the Fair Value of Stock Options and Stock Purchase Rights

The fair value of each option award is estimated on the date of grant using the Black-Scholes valuation model and the assumptions noted in the tables below. The expected life of options is based on observed historical exercise patterns. Groups of employees that have similar historical exercise patterns were considered separately for valuation purposes, but none were identified that had distinctly different exercise patterns as of March 31, 2015. The expected volatility of stock options is based upon the weighted average of the historical volatility of the Company's common stock and the implied volatility of traded options on the Company's common stock for fiscal periods in which there is sufficient trading volume in options on the Company's common stock. The risk-free interest rate is based on the implied yield on a U.S. Treasury zero-coupon issue with a remaining term equal to the expected term of the option. The dividend yield reflects that the Company has not paid any cash dividends since inception and does not intend to pay any cash dividends in the foreseeable future. The assumptions used to estimate the per share fair value of stock options granted under the 2012 Inducement Plan and 2014 Inducement Plan and the Share Incentive Plan were as follows:

	Three Months Ended March 31,	
	2015	2014
Expected volatility	44 – 45%	44 – 45%
Dividend yield	0.0%	0.0%
Expected life	7.0 years	6.9 years
Risk-free interest rate	1.5 – 2.0%	2.1 – 2.3%

During the three months ended March 31, 2015, the Company granted 642,070 options with a weighted average fair value of $56.11 per option.

The Company did not issue any new stock purchase rights under the ESPP during the three months ended March 31, 2015.

Restricted Stock Unit Awards with Service-Based Vesting Conditions

Restricted stock units (RSUs) are generally subject to forfeiture if employment terminates prior to the release of vesting restrictions. The Company expenses the cost of the RSUs, which is determined to be the fair market value of the shares of common stock underlying the RSUs at the date of grant, ratably over the period during which the vesting restrictions lapse. During the three months ended March 31, 2015, the Company granted 825,900 RSUs with a weighted average fair market value of $120.95 per share.

Restricted Stock Unit Awards with Performance and Market-Based Vesting Conditions

During 2012 and 2011, pursuant to the approval of the Board of Directors (the Board), the Company granted 860,000 RSU awards with performance and market-based vesting conditions (the 2011/2012 Base RSUs) under the Share Incentive Plan and the 2012 Inducement Plan to certain executive officers. As of March 31, 2015, the 2011/2012 Base RSUs had a weighted-average grant date fair value of $34.66. The 2011/2012 Base RSUs will vest upon the achievement of specific performance goals (the Earned RSUs). The number of RSUs that will be awarded from the Earned RSUs will be calculated by multiplying the Earned RSUs by the Total Shareholder Return multiplier which could range from 75% to 125%.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

Stock-based compensation expense for this award will be recognized over the remaining service period beginning in the period the Company determines the strategic performance goal or goals is probable of achievement. During the fourth quarter of 2014, management concluded that the achievement of the 2015 revenue performance goal was probable and began recognizing compensation expense related to the RSUs allocated to the revenue performance goal. During 2013, management concluded that regulatory approval of Vimizim was probable and began recognizing compensation expense related to the performance 2011/2012 Base RSUs allocated to the Vimizim performance goal. For the three months ended March 31, 2015 and 2014, the Company recorded $1.8 million and $0.6 million, respectively, of compensation expense related to these performance awards.

Restricted Stock Unit Awards with Performance Conditions

On March 3, 2015, pursuant to Board approval, the Company granted 58,300 RSU awards with performance-vesting conditions (the 2015 Base RSUs) under the Share Incentive Plan to certain executive officers. The vesting of the 2015 Base RSUs under this specific grant is contingent upon the achievement of a 2015 revenue target and a three-year service period. The number of RSUs that will be awarded from the 2015 Base RSUs upon achievement of the performance condition will be calculated by multiplying the 2015 Base RSUs by a revenue multiplier (determined based on the Company's performance against the revenue target) which could range between 80% or 120%. The maximum number of RSUs that could vest if the performance condition is achieved and a revenue multiplier of 120% is applied is 69,960.

Stock-based compensation for these awards will be recognized over the service period beginning in the period the Company determines it is probable that the revenue target will be achieved. The cost of the 2015 Base RSUs was determined to be $108.36 per RSU, based on the fair value of the common stock underlying the 2015 Base RSUs on the grant date. Accordingly, because the Company's management determined that attainment of the revenue target was probable as of March 31, 2015, the Company recognized $0.2 million of compensation expense related to this awards during the three months ended March 31, 2015.

Compensation expense included in the Company's Condensed Consolidated Statements of Comprehensive Loss for all stock-based compensation arrangements was as follows:

	Three Months Ended March 31,	
	2015	2014
Cost of sales	$1,348	$1,086
R&D	9,930	7,115
SG&A	11,414	8,103
Total stock-based compensation expense	$22,692	$16,304

Stock-based compensation of $2.5 million and $2.1 million was capitalized into inventory, for the three months ended March 31, 2015 and 2014, respectively. Capitalized stock-based compensation is recognized as cost of sales when the related product is sold.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(17) COMPREHENSIVE LOSS

The following table summarizes amounts reclassified out of Accumulated Other Comprehensive Income/(Loss) (AOCI) and their effect on the Company's Condensed Consolidated Statements of Comprehensive Loss for the three months ended March 31, 2015 and 2014.

	Amount Reclassified from AOCI Gain (Loss) Three Months Ended March 31,		
Details about AOCI Components	2015	2014	Consolidated Statement of Operations Classification
Gains (loss) on cash flow hedges:			
Forward foreign currency exchange contracts	$ 4,739	$ (887)	Net product revenues
	—	320	Provision for income taxes
	$ 4,739	$ (567)	Net loss

The following table summarizes changes in the accumulated balances for each component of AOCI, including current period other comprehensive income and reclassifications out of AOCI, for the three months ended March 31, 2015 and 2014.

	Three Months Ended March 31, 2015		
	Before Tax Amount	Tax (Expense) Benefit	Net-of-Tax Amount
AOCI balance at December 31, 2014	$33,984	$ (6,518)	$ 27,466
Foreign currency translation adjustment	(5)	—	(5)
Unrealized gain on available-for-sale securities:			
Unrealized holding gains	11,481	(4,160)	7,321
Less: reclassification adjustment for gain realized in net loss	—	—	—
Net unrealized holding gain	11,481	(4,160)	7,321
Net unrealized holding gain on cash flow hedges:			
Unrealized holding gain	13,776	—	13,776
Less: reclassification adjustment for gain realized in net loss	4,739	—	4,739
Net unrealized holding gain	9,037	—	9,037
Other comprehensive income	20,513	(4,160)	16,353

AOCI balance at March 31, 2015	$54,497	$ (10,678)	$ 43,819

	Three Months Ended March 31, 2014		
	Before Tax Amount	Tax (Expense) Benefit	Net-of-Tax Amount
AOCI balance at December 31, 2013	$7,756	$ (2,738)	$ 5,018
Foreign currency translation adjustment	5	—	5
Unrealized gain on available-for-sale securities:			
Unrealized holding gains	2,244	(821)	1,423
Less: reclassification adjustment for gain realized in net loss	—	—	—
Net unrealized holding gain	2,244	(821)	1,423
Net unrealized holding gain on cash flow hedges:			
Unrealized holding gain	2,183	(787)	1,396
Less: reclassification adjustment for loss realized in net loss	(887)	320	(567)
Net unrealized holding gain	1,296	(467)	829
Other comprehensive income	3,545	(1,288)	2,257
AOCI balance at March 31, 2014	$11,301	$ (4,026)	$ 7,275

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(18) REVENUE AND CREDIT CONCENTRATIONS

Net Product Revenue—The Company considers there to be revenue concentration risks for regions where net product revenue exceeds ten percent (10%) of consolidated net product revenue. The concentration of the Company's net product revenue within the regions below may have a material adverse effect on the Company's revenue and results of operations if sales in the respective regions experience difficulties.

The table below summarizes consolidated net product revenue concentrations based on patient location for Vimizim, Naglazyme, Kuvan and Firdapse and the headquarters for Genzyme Corporation (Genzyme) for Aldurazyme. Although Genzyme sells Aldurazyme worldwide, the royalties earned by the Company on Genzyme's net sales are included in the U.S. region, as the transactions are with Genzyme whose headquarters are located in the U.S.

	Three Months Ended March 31,	
	2015	2014
Region:		
United States	44 %	47 %
Europe	21 %	20 %
Latin America	17 %	16 %
Rest of world	18 %	17 %
Total net product revenue	100 %	100 %

The following table illustrates the percentage of the Company's consolidated net product revenue attributed to the Company's four largest customers.

	Three Months Ended March 31,	
	2015	2014
Customer A	15 %	16 %
Customer B [1]	9 %	12 %
Customer C	11 %	11 %
Customer D	12 %	11 %

Total	47%	50 %

(1) Genzyme is the Company's sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties. Net product revenues from Genzyme are comprised of royalties on worldwide net Aldurazyme sales and incremental product transfer revenue.

On a consolidated basis, the Company's two largest customers accounted for 29% and 21% of the March 31, 2015 accounts receivable balance, respectively, compared to December 31, 2014 when the two largest customers accounted for 42% and 18% of the accounts receivable balance, respectively. As of March 31, 2015 and December 31, 2014, accounts receivable for the Company's largest customer balance included $30.2 million and $34.5 million, respectively, of unbilled accounts receivable related to net incremental Aldurazyme product transfers to Genzyme. The Company does not require collateral from its customers, but does perform periodic credit evaluations of its customers' financial condition and requires immediate payment in certain circumstances.

The Company is subject to credit risk from accounts receivable related to product sales. The majority of the Company's trade accounts receivable arises from product sales in the U.S. and the EU. The Company's product sales to government-owned or government-funded customers in certain European countries, including Italy, Spain, Portugal, Greece and Russia, are subject to payment terms that are statutorily determined. Because these customers are government-owned or government-funded, the Company may be impacted by declines in sovereign credit ratings or sovereign defaults in these countries. A significant or further decline in sovereign credit ratings or a default in these countries may decrease the likelihood that the Company will collect accounts receivable or may increase the discount rates and the length of time until receivables are collected, which could result in a negative impact to the Company's operating results. In the three months ended March 31, 2015, the Company's net product revenues for these countries was 4%. Additionally, approximately 7% of the Company's outstanding accounts receivable at March 31, 2015 related to such countries.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

As of March 31, 2015, the Company's accounts receivable in certain European countries, specifically Greece, Italy, Portugal, Spain and Russia, totaled approximately $12.0 million, of which $0.8 million was greater than 90 days past due, $0.4 million was greater than 180 days past due and $0.1 was greater than 365 days past due.

The Company also sells its products in other countries that face economic crises and local currency devaluation. Although the Company has historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause customers in those countries to be unable to pay for the Company's products. The Company has not historically experienced a significant level of uncollected receivables and has received continued payments from its more aged accounts. The Company believes that the allowances for doubtful accounts related to these countries is adequate based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries.

(19) SEGMENT INFORMATION

The Company operates in one business segment, which primarily focuses on the development and commercialization of innovative biopharmaceuticals for serious diseases and medical conditions. All products are included in one segment because the majority of our products have similar economic and other characteristics, including the nature of the products and production processes, type of customers, distribution methods and regulatory environment.

	Three Months Ended March 31,	
	2015	2014
Net product revenue by product:		
Vimizim	$50,622	$875
Naglazyme	78,167	80,114
Kuvan	50,193	45,236
Aldurazyme	18,243	18,070
Firdapse	4,087	4,709
Total net product revenue	$201,312	$149,004

Net product revenue is based on patient location for Vimizim, Naglazyme, Kuvan and Firdapse and Genzyme's headquarters for Aldurazyme. Although Genzyme sells Aldurazyme worldwide, the royalties earned by the Company on Genzyme's net sales are included in the U.S. region, as the transactions are with Genzyme whose headquarters are located in the U.S.

The following table summarizes total revenues from external customers and collaborative partners by geographic region.

	Three Months Ended March 31,	
	2015	2014
Total revenues by geographic region:		
United States	$90,592	$71,649
Europe	41,710	30,654
Latin America	34,818	22,209
Rest of world	36,144	27,040
Total revenues	$203,264	$151,552

(20) PROVISION FOR (BENEFIT FROM) INCOME TAXES

The Company has historically computed its interim period provision for (benefit from) income taxes by applying its forecasted effective tax rate to year-to-date earnings. However, due to a significant amount of U.S. permanent differences relative to the amount of U.S. forecasted income used in computing the effective tax rate, the effective tax rate is highly sensitive to minor fluctuations in U.S forecasted income. As such, the Company computed the U.S component of the consolidated provision for (benefit from) income taxes for the three months ended March 31, 2015 and 2014 using the actual year-to-date tax calculation. Foreign tax expense was computed using a forecasted annual effective tax rate for the three months ended March 31, 2015 and 2014.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

(In thousands of U.S. dollars, except per share amounts or as otherwise disclosed)

(21) COMMITMENTS AND CONTINGENCIES

Contingencies

From time to time the Company is involved in legal actions arising in the normal course of its business. The most significant of these actions are described below.

The process of resolving matters through litigation or other means is inherently uncertain and it is possible that an unfavorable resolution of these matters could adversely affect the Company, its results of operations, financial condition and cash flows. The Company's general practice is to expense legal fees as services are rendered in connection with legal matters, and to accrue for liabilities when losses are probable and reasonably estimable.

Paragraph IV Notices

As previously disclosed, the Company received a paragraph IV notice letter, dated October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying the Company that DRL has filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of the Company's patents listed in the U.S. Food and Drug Administration's (the FDA) Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Additionally, the Company received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying the Company that Par has filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of the Company's patents listed in the FDA's Orange Book. Together with Merck & Cie (Merck), the Company filed lawsuits against both DRL and Par in the United States District Court for the District of New Jersey alleging patent infringement for the Company's patents relating to Kuvan triggering the automatic 30 month stay on the approval of each ANDA.

Contingent Payments

As of March 31, 2015, the Company is subject to contingent payments totaling approximately $733.4 million upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. Of this amount, $51.4 million relates to programs that are no longer being developed.

As of March 31, 2015, the Company has recorded $114.3 million of contingent acquisition consideration payable on its Condensed Consolidated Balance Sheet, of which $75.3 million is expected to be paid in the next twelve months.

In the normal course of business, the Company enters into various firm purchase commitments primarily related to research and development and certain inventory related items. As of March 31, 2015, these commitments for the next five years were approximately $74.0 million. These amounts primarily relate to active pharmaceutical ingredients and represent minimum purchase requirements and post marketing commitments related to the Company's approved products.

26

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

Forward-Looking Statements

This Quarterly Report on Form 10-Q contains "forward-looking statements" as defined under securities laws. Many of these statements can be identified by the use of terminology such as "believes," "expects," "anticipates," "plans," "may," "will," "projects," "continues," "estimates," "potential," "opportunity" or the negative versions of these terms and other similar expressions. These forward-looking statements may be found in "Overview," of this Item 2 and other sections of this Quarterly Report on Form 10-Q. Our actual results or experience could differ significantly from the forward-looking statements. Factors that could cause or contribute to these differences include those discussed in "Risk Factors," in Part II, Item 1A of this Quarterly Report on Form 10-Q as well as information provided elsewhere in this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2014, which was filed with the Securities and Exchange Commission (the SEC) on March 2, 2015. You should carefully consider that information before you make an investment decision.

You should not place undue reliance on these types of forward-looking statements, which speak only as of the date that they were made. These forward-looking statements are based on the beliefs and assumptions of our management based on information currently available to management and should be considered in connection with any written or oral forward-looking statements that we may issue in the future as well as other cautionary statements we have made and may make. We do not undertake any obligation to release publicly any revisions to these forward-looking statements after completion of the filing of this Quarterly Report on Form 10-Q to reflect later events or circumstances or the occurrence of unanticipated events.

The following discussion of our financial condition and results of operations should be read in conjunction with our Condensed Consolidated Financial Statements and the related Notes thereto included elsewhere in this Quarterly Report on Form 10-Q.

Overview

We develop and commercialize innovative biopharmaceuticals for serious diseases and medical conditions. We select product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products.

Our product portfolio is comprised of five approved products and multiple clinical and pre-clinical product candidates. Our approved products are Vimizim (elosulfase alpha), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

Business Highlights

During the first quarter of 2015, we continued to grow our commercial business and advance our product pipeline. We believe that the combination of our internal research programs, acquisition and partnerships will allow us to continue develop and commercialize innovative biopharmaceuticals for serious diseases and medical conditions. Below is a summary of our recent key accomplishments:

- We acquired Prosensa Holding N.V. (Prosensa) for a total purchase price of $751.5 million. See Note 5 to our accompanying Condensed Consolidated Financial Statements for additional discussion.

- We announced the completion of a United States (U.S) Food and Drug Administration (the FDA) New Drug Application (NDA) for drisapersen and we expect to file a Marketing Authorization Application with the European Medicines Agency by the end of the third quarter 2015;

- We reported total revenues of $203.3 million for the three months ended March 31, 2015 as compared to $151.6 million for the three months ended March 31, 2014.

- We announced the selection of BMN 270, an AAV VIII vector, a Factor VIII gene therapy drug development candidate, for the treatment of hemophilia A. We submitted a clinical trial application (CTA) for a Phase 1/2 trial of BMN 270 in March 2015 and expect to initiate the study in the second half of 2015;

- We shared the interim data from nine patients in the cerliponase alpha trial who have been followed for at least 15 months. Preliminary data suggest that treatment with cerliponase alpha may result in stabilization of the disease compared to the natural history based on a standardized measure of motor and language function. We expect to release the top line results from this Phase 1/2 trial in the fourth quarter of 2015; and

Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

· We continued the treatment of the first three cohorts of the Phase 2 trial of BMN 111 for the treatment of achondroplasia. We expect to release the top line results from the three cohorts in the second quarter of 2015.

Financial Highlights

Key components of our results of operations include the following (in millions):

	Three Months Ended March 31,	
	2015	2014
Total net product revenues	$201.3	$149.0
Cost of sales	32.8	22.8
Research & Development (R&D) expense	142.1	86.2
Selling, general and administrative (SG&A) expense	92.8	60.1
Net loss	(67.5)	(38.1)
Stock-based compensation expense	22.7	16.3

See "Results of Operations" below for a discussion of the detailed components and analysis of the amounts above.

Net product revenues were as follows (in millions):

	Three Months Ended March 31,	
	2015	2014
Vimizim	$50.6	$0.9
Naglazyme	78.2	80.1
Kuvan	50.2	45.2
Aldurazyme	18.2	18.1
Firdapse	4.1	4.7
Total net product revenues	$201.3	$149.0

Cost of sales includes raw materials, personnel and facility and other costs associated with manufacturing Vimizim, Naglazyme and Aldurazyme at our production facility in Novato, California. Cost of sales also includes third-party manufacturing costs for the production of the active ingredient in Kuvan and Firdapse and third-party production costs related to final formulation and packaging services for all products and cost of royalties payable to third-parties for all products.

R&D expense includes costs associated with the research and development of product candidates and post-marketing research commitments related to our approved products. These costs primarily include preclinical and clinical studies, personnel and raw materials costs associated with manufacturing product candidates, quality control and assurance, research and development facilities and regulatory costs.

SG&A expense primarily includes expenses associated with the commercialization of approved products and general and administrative costs to support our operations. These expenses include: product marketing and sales operations personnel; corporate facility operating expenses; information technology expenses and depreciation; and core corporate support functions, including human resources, finance and legal, and other external corporate costs such as

insurance, legal fees and other professional services.

Our cash, cash equivalents, short-term investments and long-term investments totaled $1,232.6 million as of March 31, 2015, compared to $1,043.1 million as of December 31, 2014. We have historically financed our operations primarily through our cash flows from operating activities and the issuance of common stock and convertible debt. We will be highly dependent on our net product revenue to supplement our current liquidity and fund our operations for the foreseeable future. We may in the future elect to supplement this with further debt or equity offerings or commercial borrowing, even after giving effect to our January 2015 equity offering. Further, depending on market conditions, our financial position and performance and other factors, we may in the future choose to use a portion of our cash or cash equivalents to repurchase our convertible debt or other securities. See “Financial Position, Liquidity and Capital Resources” below for a further discussion of our liquidity and capital resources.

Critical Accounting Policies and Estimates

In preparing our Condensed Consolidated Financial Statements in accordance with accounting principles generally accepted in the U.S. and pursuant to the rules and regulations promulgated by the SEC, we make assumptions, judgments and estimates that can have a significant impact on our net income/loss and affect the reported amounts of certain assets, liabilities, revenue and expenses, and related disclosures. We base our assumptions, judgments and estimates on historical experience and various other factors that we believe to be reasonable under the circumstances. Actual results could differ materially from these estimates under different assumptions or conditions. On a regular basis, we evaluate our assumptions, judgments and estimates. We also discuss our critical accounting policies and estimates with the Audit Committee of our Board of Directors.

We believe that the assumptions, judgments and estimates involved in the accounting for business combinations, contingent acquisition consideration payable, income taxes, long-lived assets, revenue recognition and inventory have the greatest impact on our Condensed Consolidated Financial Statements, so we consider these to be our critical accounting policies. Historically, our assumptions, judgments and estimates relative to our critical accounting policies have not differed materially from actual results.

There have been no significant changes to our critical accounting policies and estimates during three months ended March 31, 2015, as compared to the critical accounting policies and estimates disclosed in "Management's Discussion and Analysis of Financial Condition and Results of Operations" included in our Annual Report on Form 10-K for the year ended December 31, 2014.

Recent Accounting Pronouncements

See Note 4 to our accompanying Condensed Consolidated Financial Statements for a full description of recent accounting pronouncements and our expectation of their impact, if any, on our results of operations and financial condition.

Results of Operations

Net Loss

Our net loss for the three months ended March 31, 2015 was $67.5 million, compared to a net loss of $38.1 million for the three months ended March 31, 2014. The increase in net loss was primarily a result of the following (in millions):

	Three Months
Net loss for the period ended March 31, 2014	$ (38.1)
Increased R&D expense	(55.9)
Increased SG&A expense	(32.7)
Increased gross profit from product sales	42.3
Increased benefit from income taxes	10.7
Other individually insignificant fluctuations	6.2
Net loss for the period ended March 31, 2015	$ (67.5)

The increase in R&D expense was primarily attributed to the clinical trials of our late-stage development programs, licensing fees paid to a third-party to secure licenses related to the development of talazoparib and increased research on earlier stage development programs. The increase in SG&A expense was primarily due to increased sales and

marketing expenses related to our commercial products and increased expenses related to the commercial launch of Vimizim. The increase in gross profit from product sales was primarily a result of the commercial launch of Vimizim and additional Kuvan patients initiating therapy in the U.S.

See below for additional information related to the primary net loss fluctuations presented above, including details of our operating expense fluctuations.

Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

Net Product Revenues, Cost of Sales and Gross Profit

Net product revenues by product were as follows (in millions):

	Three Months Ended March 31,		
	2015	2014	Change
Vimizim	$50.6	$0.9	$ 49.7
Naglazyme	78.2	80.1	(1.9)
Kuvan	50.2	45.2	5.0
Aldurazyme	18.2	18.1	0.1
Firdapse	4.1	4.7	(0.6)
Total net product revenues	$201.3	$149.0	$ 52.3

Net product revenues attributed to our collaboration with Genzyme Corporation (Genzyme) were as follows (in millions):

	Three Months Ended March 31,		
	2015	2014	Change
Aldurazyme revenue reported by Genzyme	$53.4	$55.9	$ (2.5)

	Three Months Ended March 31,		
	2015	2014	Change
Royalties earned from Genzyme	$22.3	$21.9	$ 0.4
Incremental (previously recognized) Aldurazyme product transfer revenue	(4.1)	(3.8)	(0.3)
Total Aldurazyme net product revenues	$18.2	$18.1	$ 0.1

The FDA and the European Medicines Agency (the EMA) granted marketing approval for Vimizim in February 2014 and April 2014, respectively, and Vimizim subsequently received marketing approval in other countries. We began marketing Vimizim immediately following approval in each of these markets. Net product revenues for Vimizim for the three months ended March 31, 2015 totaled $50.6 million, of which $34.6 million was earned from customers based outside the U.S., compared to $0.9 million for the three months ended March 31, 2014, of which $0.4 was earned for customers based outside the U.S. The increase in Vimizim net product revenues for the three months ended March 31, 2015 was attributed to new patients initiating therapy. The impact of foreign currency exchange rates on Vimizim sales denominated in currencies other than the U.S. dollar was negative by $3.9 million for the three months ended March 31, 2015. Vimizim gross margins were 85% and 91% for the three months ended March 31, 2015 and 2014, respectively. In future periods, we expect Vimizim gross margins to decline and approximate Naglazyme gross margins as we deplete previously expensed product.

Net product revenues for Naglazyme for the three months ended March 31, 2015 totaled $78.2 million, of which $68.5 million was earned from customers based outside the U.S., compared to $80.1 million for the three months ended March 31, 2014, of which $70.7 million was earned from customers based outside the U.S. The decrease in Naglazyme net product revenues for the three months ended March 31, 2015 was attributed was primarily attributed to the impact of foreign currency exchange rates on Naglazyme sales denominated in currencies other than the U.S. dollar which was negative by $3.0 million for the three months ended March 31, 2015 compared to a positive impact of $0.1 million for the three months ended March 31, 2014. Naglazyme gross margins were 85% and 86% for the three months ended March 31, 2015 and 2014, respectively. Naglazyme gross margins are not expected to fluctuate significantly in the future.

Net product revenue for Kuvan for the three months ended March 31, 2015 totaled $50.2 million, compared to $45.2 million for the three months ended March 31, 2014. The increase in Kuvan net product revenues was attributed to new patients initiating therapy. Kuvan gross margins were 82% and 84% for the three months ended March 31, 2015 and 2014, respectively. Cost of goods sold for each of the three months ended March 31, 2015 and 2014 reflect royalties paid to third-parties of approximately 10%. Kuvan gross margins are not expected to fluctuate significantly in the future. The royalties earned from Merck Serono’s net sales of Kuvan for each of the three months ended March 31, 2015 and 2014 were $0.5 million.

We own several patents that cover Kuvan and have listed those patents in conjunction with that product in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). The Drug Price Completion and Patent Term Restoration Act of 1984, known as the Hatch-Waxman Act permits the FDA to approve abbreviated new drug applications (ANDA), for the generic versions of branded drugs. The Hatch-Waxman Act requires an ANDA applicant seeking FDA approval of the applicant's proposed generic product prior to the expiration of our Orange Book-listed patents to notify us of the application. Upon receipt of such a notice (a paragraph IV notice), the Hatch-Waxman Act allows us, with proper basis, to bring an action for patent infringement against the ANDA filer, asking that the proposed generic product not be approved until after our patents expire. If we commence a lawsuit within 45 days from receipt of the paragraph IV notice, the Hatch-Waxman Act provides a 30-month stay, during which time the FDA cannot finally approve the application for the generic version of our drug. If the litigation is resolved in favor of the ANDA applicant during the 30-month stay period, the stay is lifted and the FDA's review of the ANDA may be completed. The discovery, trial and appeals process in such a lawsuit is costly, time consuming, and may result in generic competition if the ANDA applicant prevails. Regardless of any litigation results, generic versions of Kuvan would be prohibited until the expiration of orphan drug exclusivity in June 2015, including pediatric exclusivity, at the earliest. We have also received New Patient Population exclusivity for Kuvan (sapropterin dihydrochloride) that expires in October 2017, including pediatric exclusivity. Thus, depending on the label of a generic product, generic versions of Kuvan may be prohibited until October 2017.

As previously disclosed, we have received a paragraph IV notice letter, dated October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying us that DRL has filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Additionally, we have received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying us that Par has filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Together with Merck & Cie (Merck), we filed lawsuits against both DRL and Par in the United States District Court for the District of New Jersey alleging patent infringement for our patents relating to Kuvan triggering the automatic 30 month stay on the approval of each ANDA.

The filing of DRL's and Par's purported ANDAs in respect to Kuvan (sapropterin dihydrochloride) could have an adverse impact on our stock price, and litigation to enforce our patents is likely to cost a substantial amount and require significant management attention. If the patents covering Kuvan (sapropterin dihydrochloride) and its use are not upheld in litigation, or if DRL and/or Par is found to not infringe our asserted patents, the resulting generic competition following the expiration of regulatory exclusivity would have a material adverse effect on our revenue and results of operations.

Net product revenue for Aldurazyme for the three months ended March 31, 2015 totaled $18.2 million, compared to $18.1 million for the three months ended March 31, 2014. Aldurazyme gross margins were 79% for the three months ended March 31, 2015, compared to 82% for the three months ended March 31, 2014. Aldurazyme gross margins reflect the profit earned on royalty revenue and net incremental product transfer revenue. Aldurazyme gross margins are expected to fluctuate depending on the mix of royalty revenue, from which we earn higher gross profit, and product transfer revenue, from which we earn lower gross profit.

Net product revenue for Firdapse for the three months ended March 31, 2015 totaled $4.1 million, compared to $4.7 million for the three months ended March 31, 2014. The decrease in Firdapse net product revenues was attributed to the negative impact of foreign exchange rates for the Euro. Firdapse gross margins for the three months ended March 31, 2015 and 2014 were 78% and 75%, respectively. Cost of goods sold for the each of the three months ended March

31, 2015 and 2014 reflect royalties paid to third-parties of approximately 8%. Firdapse gross margins are not expected to increase over the next twelve months due to higher production yields and normalize in the mid-eighties.

Cost of Sales

Total cost of sales for the three months ended March 31, 2015 were $32.8 million, compared to $22.8 million for the three months ended March 31, 2014. The increase in cost of sales was primarily attributed to the increase in product sales.

Research and Development

We manage our R&D expense by identifying the research and development activities we anticipate will be performed during a given period and then prioritizing efforts based on scientific data, probability of successful development, market potential, available human and capital resources and other similar considerations. We continually review our pipeline and the development status of product candidates and, as necessary, reallocate resources among the research and development portfolio that we believe will best support the future growth of our business.

R&D expense increased to $142.1 million for the three months ended March 31, 2015, from $86.2 million for the three months ended March 31, 2014. The increase in R&D expense was primarily a result of the following (in millions):

	Three Months
R&D expense for the period ended March 31, 2014	$ 86.2
Increased talazoparib development expense	13.3
Increased development expense on early development stage programs	8.0
Increased reveglucosidase alfa development expense	5.8
Drisapersen development expense	6.4
Increased BMN 111 development expense	4.4
Increased cerliponase alfa development expense	4.1
Decreased Vimizim development expense	(5.5)
Increased non-allocated R&D expense and other net changes	19.4
R&D expense for the period ended March 31, 2015	$ 142.1

The increase in talazoparib, reveglucosidase alfa, BMN 111 and cerliponase alfa development expense was attributed to increased clinical trial activities related to these product candidates. The increase in development expense on early development stage programs was primarily attributed to the pre-clinical activity related to BMN 270 and BMN 250 and development costs related to the programs acquired from Zacharon Pharmaceuticals, Inc. (Zacharon). The development expenses for drisapersen relate to clinical activities for the product candidate that was acquired from Prosensa in January 2015. The increase in non-allocated R&D expense is primarily attributed to an increase in R&D personnel costs and facility costs that are not allocated to specific programs. Non-allocated R&D expense for the three months ended March 31, 2014, included a $6.1 million gain on early lease termination of our San Rafael Corporate Center (SRCC) lease resulting from the recognition of the remaining deferred rent and asset retirement liabilities upon acquisition of SRCC. There was no similar gain during the three months ended March 31, 2015.

During the remainder of 2015, we expect our R&D spending to increase over 2014 levels due to our drisapersen, pegvaliase, talazoparib, reveglucosidase alfa, BMN 111 and cerliponase alfa programs progressing, including a few of those programs progressing to more advanced phases of clinical studies. We acquired drisapersen, which is in Phase 3 clinical trials, in January 2015 from Prosensa. Phase 3 clinical trials for pegvaliase and talazoparib were initiated in the second and fourth quarters of 2013, respectively, and we initiated a Phase 3 trial of reveglucosidase alfa in the second quarter of 2014. We also expect increased spending on pre-clinical and clinical activities for our early development stage programs including BMN 270, BMN 250 and programs acquired from Zacharon. Additionally, we expect to continue incurring significant R&D expense for the foreseeable future due to long-term clinical activities related to post-approval regulatory commitments for our approved products. We continuously evaluate the recoverability of costs associated with pre-launch manufacturing activities, and if it is determined that recoverability is highly likely and therefore future revenues are expected, the costs subsequently incurred related to pre-launch manufacturing activities may be capitalized. When regulatory approval and the likelihood of future revenues for a

product candidate are less certain, the related manufacturing costs are expensed as R&D expenses.

Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

Selling, General and Administrative

SG&A expense increased to $92.8 million for the three months ended March 31, 2015, from $60.1 million for the three months ended March 31, 2014. The increase in SG&A expense was primarily a result of the following (in millions):

	Three Months
SG&A expense for the period ended March 31, 2014	$ 60.1
Increased Vimizim commercial launch expenses	4.3
Business acquisition transaction costs	7.0
Net increase in corporate support and other administrative expenses	21.4
SG&A for the period ended March 31, 2015	$ 92.8

We received regulatory approval to market Vimizim in the U.S. and the EU during 2014. The increase in commercial launch expense is consistent with the timing of these approvals. The increase in corporate support and other administrative expenses is primarily attributed to increases in employee-related expenses due to the increase in commercial and administrative headcount, stock based compensation, consulting fees, and information technology expenses. Corporate support and administrative expenses for the three months ended March 31, 2014, included a $2.7 million gain on early lease termination of our SRCC lease resulting from the recognition of the remaining deferred rent and asset retirement liabilities upon acquisition of the SRCC, which is where our corporate headquarters are located. There was no similar gain during the three months ended March 31, 2015. We expect SG&A expense to increase in future periods as a result of the international expansion of Naglazyme and Vimizim, the U.S. commercialization activities for Kuvan, and the increase in administrative support required for our expanding operations.

Intangible Asset Amortization and Contingent Consideration

Intangible asset amortization and contingent consideration expense is comprised of changes in the fair value of contingent acquisition consideration payable to former stockholders of our acquired businesses, impairment loss (if any) on intangible assets and amortization of intangible assets. Changes in the fair value of contingent acquisition consideration payable result from updates to the estimated probability of achievement or assumed timing of milestones and adjustments to the discount periods and rates. Intangible asset amortization and contingent consideration expense consisted of the following (in millions):

	Three Months Ended March 31,		
	2015	2014	Change
Changes in the fair value of contingent acquisition consideration payable	$0.3	$8.2	$ (7.9)
Amortization of intangible assets	1.1	0.8	0.3
Total intangible asset amortization and contingent	$1.4	$9.0	$ (7.6)

consideration

The changes in the fair value of the contingent acquisition consideration payable were primarily attributed to changes in the estimated probability of achieving development milestones based on the current status of the related development programs as well as changes in the discount rate utilized in the fair value calculations. During the three months ended March 31, 2014, the majority of the changes related to the development progress of reveglucosidase alfa for which the contingent consideration expense was $6.0 million.

Interest Income

We invest our cash, short-term and long-term investments in government and other high credit quality securities in order to limit default and market risk. Interest income totaled $0.7 million for the three months ended March 31, 2015, compared to $1.1 million for the three months ended March 31, 2014. We do not expect future interest income to fluctuate significantly over the next twelve months.

Interest Expense and Debt Conversion Expense

We incur interest expense on our convertible debt and our capital leases. Interest expense consisted of the following (in millions):

	Three Months Ended March 31,		
	2015	2014	Change
Coupon interest	$2.5	$2.4	$ 0.1
Amortization of issuance costs	0.8	0.8	—
Accretion of discount on convertible notes	6.2	5.9	0.3
Total interest expense	$9.5	$9.1	$ 0.4

The interest expense is primarily attributed to amounts related to our October 2013 issuance of $750.0 million in aggregate principal amount of senior subordinated convertible debt. We do not expect future interest expense to fluctuate significantly over the next twelve months. See Note 13 to the accompanying Condensed Consolidated Financial Statements for additional information regarding our Convertible Debt.

During the three months ended March 31, 2015, we recognized Debt Conversion Expense of $0.2 million in connection with the early conversion of $8.1 million in aggregate principal amount of our senior subordinated convertible notes due in 2017 (the 2017 Notes).

Provision for (Benefit from) Income Taxes

For the three months ended March 31, 2015 we recognized a benefit from income taxes of $7.2 million, compared to the three months ended March 31, 2014 when we recognized income tax expense of $3.5 million. We have historically computed interim period provision for (benefit from) income taxes by applying our forecasted effective tax rate to year-to-date earnings. However, due to a significant amount of U.S. permanent differences relative to the amount of U.S. forecasted income in computing the effective tax rate, the effective tax rate is highly sensitive to minor fluctuations in the U.S. forecasted income. As such, we have computed U.S. component of the provision for (benefit from) income taxes for the three months ended March 31, 2015 and 2014 using an actual year-to-date tax calculation. Foreign tax expense was computed using a forecasted annual effective tax rate for the three months ended March 31, 2015 and 2014.

The provision for (benefit from) income taxes for the three months ended March 31, 2015 and 2014, consisted of state, federal and foreign current tax expense which was offset by deferred tax benefits from federal orphan drug credits and California R&D credits. During the three months ended March 31, 2015, increased R&D expense attributed to an increase in expenses which qualified for the federal orphan drug and California R&D credits as compared to the three months ended March 31, 2014. The provisions for the three months ended March 31, 2015 and

2014 were further reduced by the tax benefit related to stock option exercises during these periods. See Note 15 to our Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2014 for additional discussion of the components of our provision for (benefit from) income taxes.

Management's Discussion and Analysis of Financial Condition and Results of Operations – (Continued)

Financial Position, Liquidity and Capital Resources

We expect to fund our operations with our net product revenues from our commercial products, cash, cash equivalents, short-term and long-term investments supplemented by proceeds from equity or debt financings and loans or collaborative agreements with corporate partners, each to the extent necessary. This expectation could change depending on how much we elect to spend on our development programs, potential licenses, and acquisitions of complementary technologies, products and companies or if we elect to settle all or a portion of our convertible debt in cash. We will be highly dependent on our net product revenue to supplement our current liquidity and fund our operations for the foreseeable future. We may in the future elect to supplement this with further debt or equity offerings or commercial borrowing, even after giving effect to our October 2013 debt offering and our March 2014 and January 2015 equity offerings.

We consider the unrepatriated cumulative earnings of certain of our foreign subsidiaries to be indefinitely invested outside the U.S. As of March 31, 2015, $227.1 million of our $1,232.6 million balance of cash, cash equivalents and marketable securities was held in foreign subsidiaries, a significant portion of which is required to fund the liquidity needs of these foreign subsidiaries. In managing our liquidity needs in the U.S., we do not rely on the unrepatriated earnings as a source of funds and we have not provided for U.S. federal or state income taxes on these undistributed foreign earnings.

We are mindful that conditions in the current macroeconomic environment could affect our ability to achieve our goals. Some of the factors that could affect our business include: future changes to healthcare reform in the U.S., a continuation or worsening of global economic conditions, patent expirations of competitive products and the launch of generic competitors, continued government pricing pressures internationally and the potential volatility in foreign currency exchange rates. We will continue to monitor these conditions and will attempt to adjust our business processes, as appropriate, to mitigate these risks to our business.

Our financial condition as of March 31, 2015 and December 31, 2014 was as follows (in millions):

	March 31, 2015	December 31, 2014	Change
Cash and cash equivalents	$900.6	$875.5	$25.1
Short-term investments	108.1	69.7	38.4
Long-term investments	223.9	97.9	126.0
Cash, cash equivalents and investments	$1,232.6	$1,043.1	$189.5
Current assets	$1,522.7	$1,432.2	$90.5
Current liabilities	305.5	235.7	69.8
Working capital	$1,217.2	$1,196.5	$20.7
Convertible debt	$655.5	$658.0	$(2.5)

Our cash flows for each of the three months ended March 31, 2015 and 2014 are summarized as follows (in millions):

	2015	2014	Change
Cash and cash equivalents at the beginning of the period	$875.5	$568.8	$306.7
Net cash used in operating activities	(137.8)	(26.5)	(111.3)
Net cash used in investing activities	(750.9)	(38.5)	(712.4)
Net cash provided by financing activities	914.8	137.0	777.8
Foreign exchange impact	(1.0)	(1.0)	—
Cash and cash equivalents at the end of the period	$900.6	$639.8	$260.8
Short-term and long-term investments	332.0	499.1	(167.1)
Cash, cash equivalents and investments	$1,232.6	$1,138.9	$93.7

Working Capital

Working capital increased by $20.7 million, from $1,196.5 million at December 31, 2014 to $1,217.2 million at March 31, 2015. The increase in working capital was attributed to the following (in millions):

Working capital at December 31, 2014	$1,196.5
Increased cash, cash equivalents and short-term investments	63.5
Increased accounts receivable, net	31.3
Increased inventory	23.4
Increased current liabilities	(69.9)
Decreased other current assets	(27.6)
Working capital at March 31, 2015	$1,217.2

The increase in cash, cash equivalents and short-term investments at March 31, 2015 from December 31, 2014 was primarily attributed to the net proceeds of $888.3 million from our January 2015 public offering of common stock, proceeds of $28.0 million from employee stock option exercises and employee stock purchase plan (ESPP) contributions. The increase in accounts receivable is attributed to increased revenues and the timing of net product revenues and cash receipts from customers. The increase in inventory was primarily attributed to building of inventories for all commercial products to meet anticipated future sales demand.

Our product sales to government-owned or government-funded customers in certain countries, including Russia, Greece, Spain, Italy and Portugal, are subject to payment terms that are imposed by government authority. Because these customers are government-owned or government-funded, we may be impacted by declines in sovereign credit ratings or sovereign defaults in these countries. A significant or further decline in sovereign credit ratings, or default in these countries, may decrease the likelihood that we will collect accounts receivable or may increase the discount rates and the length of time until receivables are collected, which could result in a negative impact to our operating results. Historically we have not experienced a significant level of uncollected receivables and have received continued payments from our more aged accounts. We believe that the allowances for doubtful accounts for these countries are adequate based on our analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries. As of March 31, 2015, approximately 7% of our outstanding accounts receivable relate to such countries. See Note 18 to our accompanying Condensed Consolidated Financial Statements for additional discussion. We also sell our products in other countries that face economic crises and local currency devaluation. Although we have historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause our customers in those countries to be unable to pay for our products with the same negative effect on our operations.

Cash Used in Operating Activities

Cash used in operating activities for the three months ended March 31, 2015 was $137.8 million, compared to cash used in operating activities of $26.5 million for the three months ended March 31, 2014. The increase in cash used in operating activities was primarily attributed to the $29.4 million increase in our net loss, a $34.2 million decrease in collection of accounts receivable and a $10.1 million increase in inventory purchases. The increase in our net loss is primarily attributed to increased R&D expense related to increased clinical trial activities for our product candidates: talazoparib, reveglucosidase alfa, cerliponase alfa and drisapersen and increased sales and marketing expense related to the commercial launch of Vimizim in the U.S. and the EU.

Cash Used in Investing Activities

Net cash used in investing activities during the three months ended March 31, 2015 and 2014 was $750.9 million and $38.5 million, respectively. Our investing activities have consisted primarily of purchases and sales and maturities of investments and capital expenditures, such as manufacturing equipment and facility construction and improvements. The increase in net cash used by investing activities for the three months ended March 31, 2015 compared to the three months ended March 31, 2014 was primarily comprised of a $538.4 million increase due to the Prosensa acquisition, net of cash acquired, a $20.2 million increase in capital expenditures and an increase of $149.4 million in net purchases of investments. During the remainder of 2015, we expect to make significant capital investments in our Shanbally, Ireland manufacturing facility to enable future commercial manufacturing of our products at the facility and our corporate headquarters at SRCC to accommodate additional laboratory space requirements and anticipated headcount growth.

Cash Provided by Financing Activities

Net cash provided by financing activities for the three months ended March 31, 2015 was $914.8 million, compared to net cash provided by financing activities of $137.0 million for the three months ended March 31, 2014. Historically, our financing activities primarily included proceeds from the sale of our convertible debt, common stock and employee stock purchases under the ESPP and employee stock option exercises, offset by payments related to our contingent acquisition obligations and convertible debt obligations. The increase in net cash provided by financing activities for the three months ended March 31, 2015 was primarily attributed to a $770.8 million increase in net proceeds from our January 2015 equity offering compared to the March 2014 equity offering and increased proceeds from employee stock option exercises and ESPP contributions of $8.3 million.

Other Information

On January 15, 2015, we closed the initial offering period related to our offer to purchase all of the ordinary shares (Prosensa Shares) of Prosensa Holding N.V. (Prosensa), a public limited liability company organized under the laws of the Netherlands, purchasing 93.4% of the Prosensa Shares and immediately launched a subsequent offering period that expired on January 29, 2015. As of the expiration of the subsequent offering period we paid approximately $620.7 million for approximately 35 million Prosensa Shares, representing approximately 96.8% of all the outstanding Prosensa Shares. Additionally, we paid approximately $38.6 million for the options that vested pursuant to the definitive purchase agreement. On February 12, 2015, we completed an asset transfer and we paid $20.8 million to the remaining Prosensa shareholders. Effective February 12, 2015, Prosensa has been dissolved and is in liquidation under Dutch law.

On January 27, 2015, we sold approximately 9.8 million shares of our common stock at a price of $93.25 per share in an underwritten public offering pursuant to an effective registration statement previously filed with the SEC. We received net proceeds of approximately $888.3 million from this public offering after underwriter's discount and offering costs.

On October 15, 2013, we completed an offering of $750.0 million in aggregate principal amount of senior subordinated convertible notes consisting of $375.0 million in aggregate principal amount of 0.75% senior subordinated convertible notes due 2018 (the 2018 Notes) and $375.0 million in aggregate principal amount of 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes and together with the 2018 Notes, the Notes). The net proceeds from the offering were $696.4 million, after deducting commissions and offering expenses and the purchase of capped calls. The Notes were issued at face value and accrue interest at their stated annual rates which is payable semiannually in arrears on April 15 and October 15 of each year beginning on April 15, 2014. See Note 13 to our accompanying Condensed Consolidated Financial Statements for additional discussion.

In April 2007, we sold approximately $324.9 million of the 2017 Notes of which $31.9 million remained outstanding at March 31, 2015. The debt was issued at face value and bears interest at the rate of 1.875% per annum, payable semi-annually in cash. During the three months ended March 31, 2015, we entered into separate agreements with three of the existing holders of the 2017 Notes pursuant to which such holder converted $8.1 million in aggregate principal amount of the 2017 Notes into 399,469 shares of our common stock. In addition to issuing the requisite number of shares of common stock, we the also made varying cash payments to the holders totaling $0.2 million in aggregate, of which $0.2 million was recognized in total as Debt Conversion Expense on the Condensed Consolidated Statement of Comprehensive Loss for the three months ended March 31, 2015.

Our $781.9 million (undiscounted) of total convertible debt as of March 31, 2015 will impact our liquidity due to the semi-annual cash interest payments and will further impact our liquidity if we elect to settle all or portions of the 2018 Notes or the 2020 Notes in cash upon conversion or if the holders of our 2017 Notes do not convert on or prior to the scheduled repayments of the debt. Further, depending on market conditions, our financial position and performance and other factors, we may in the future choose to use a portion of our cash or cash equivalents to repurchase our convertible debt or other securities.

Funding Commitments

We cannot estimate with certainty the cost to complete any of our product development programs. Additionally, except as disclosed under "Overview" above, we cannot precisely estimate the time to complete any of our product development programs or when we expect to receive net cash inflows from any of our product development programs. Please see "Risk Factors" included in Part II, Item 1A of this Quarterly Report on Form 10-Q, for a discussion of the reasons we are unable to estimate such information, and in particular the following risk factors:

- If we fail to obtain or maintain regulatory approval to commercially market and sell our drugs, or if approval is delayed, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased;

- If we are unable to successfully develop and maintain manufacturing processes for our drug products to produce sufficient quantities at acceptable costs, we may be unable to meet demand for our products and lose potential revenue, have reduced margins or be forced to terminate a program;

- If we fail to compete successfully with respect to product sales, we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product and our revenue could be adversely affected; and

- If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

Our investment in our product development programs and continued development of our existing commercial products has a major impact on our operating performance. Our R&D expenses in each of the three months ended March 31, 2015 and 2014 and the period since inception of the major programs were as follows (in millions):

	Three Months Ended March 31,		Since Program Inception
	2015	2014	
Vimizim	$12.5	$18.0	$ 369.9
Talazoparib (BMN 673)	18.3	5.0	134.7
Reveglucosidase alfa (BMN 701)	16.6	10.8	164.9
BMN 111	8.7	4.3	78.1
Cerliponase alfa (BMN 190)	9.2	5.1	80.3
Pegvaliase (PEG PAL)	16.7	16.0	254.9
Drisapersen	6.4	—	6.4
Mature approved products	8.1	7.1	411.4
Not allocated to specific major current projects	45.6	19.9	Not meaningful
Totals	$142.1	$86.2	

We may elect to increase our spending above our current long-term plans and consequently we may be unable to achieve our long-term goals. This may increase our capital requirements, including: costs associated with the commercialization of our products; additional clinical trials; investments in the manufacturing of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse; preclinical studies and clinical trials for our other product candidates; potential licenses and other acquisitions of complementary technologies, products and companies; and general corporate purposes.

Our future capital requirements will depend on many factors, including, but not limited to:

· product sales and profitability of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;

· manufacture, supply or distribution of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;

· progress of our integration of Prosensa;

- progress of our product candidates through the regulatory process and our ability to successfully commercialize any such products that receive regulatory approval;

- results of clinical trials, announcements of technological innovations or new products by us or our competitors;

- results relating to our lawsuit against DRL to protect our patents relating to Kuvan;

- government regulatory action affecting our product candidates or our competitors' drug products in both the U.S. and non-U.S. countries;

- developments or disputes concerning patent or proprietary rights;

- general market conditions and fluctuations for the emerging growth and pharmaceutical market sectors;

- economic conditions in the U.S. or abroad;

- broad market fluctuations in the U.S., the EU or in other parts of the world;

- actual or anticipated fluctuations in our operating results; and

- Changes in company assessments or financial estimates by securities analysts.

Off-Balance Sheet Arrangements

We do not have any off-balance sheet arrangements that are currently material or reasonably likely to be material to our consolidated financial position or results of operations.

Contractual and Commercial Obligations

We have contractual and commercial obligations under our debt, operating leases and other obligations related to R&D activities, purchase commitments, licenses and sales royalties with annual minimums. Our contractual obligations for non-cancelable purchase commitments as of March 31, 2015 are presented in the table below (in millions).

	Payments Due within			
		>1 -3	> 3 - 5	More Than 5

	1 Year or Less	Years	Years	Years	Total
2017 Notes and related interest	$0.6	$32.8	$—	$—	$33.4
2018 Notes and related interest	2.8	5.6	377.8	—	386.2
2020 Notes and related interest	5.6	11.2	11.6	380.6	409.0
Operating leases	4.9	13.6	2.4	0.1	21.0
R&D and purchase commitments	47.2	24.6	2.2	—	74.0
Total	$61.1	$87.8	$394.0	$380.7	$923.6

We are also subject to contingent payments totaling approximately $733.4 million as of March 31, 2015 which are due upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. Of this amount, $51.4 million relates to programs that are no longer being developed.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Our market risks during the three months ended March 31, 2015 have not materially changed from those discussed in Part II, Item 7A of our Annual Report on Form 10-K for the year ended December 31, 2014, which was filed with the SEC on March 2, 2015.

Item 4. Controls and Procedures

(a) Controls and Procedures

An evaluation was carried out, under the supervision of and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the Exchange Act), as of the end of the period covered by this report.

Based on the evaluation, our Chief Executive Officer and our Chief Financial Officer have concluded that our disclosure controls and procedures are effective to ensure that the information required to be disclosed by us in the reports we file or submit under the Exchange Act was recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms.

(b) Change in Internal Controls over Financial Reporting

There were no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act, during our most recently completed quarter that have materially affected or are reasonably likely to materially affect our internal control over financial reporting. We are utilizing the Committee of Sponsoring Organizations of the Treadway Commission (COSO) 2013 Framework on internal control.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings.

Paragraph IV Notices

As previously disclosed, we have received a paragraph IV notice letter, dated October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying us that DRL has filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the U.S. Food and Drug Administration's (the FDA) Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Additionally, we have received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying us that Par has filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Together with Merck & Cie (Merck), we filed lawsuits against both DRL and Par in the United States District Court for the District of New Jersey alleging patent infringement for our patents relating to Kuvan triggering the automatic 30 month stay on the approval of each ANDA.

Item 1A. Risk Factors

An investment in our securities involves a high degree of risk. We operate in a dynamic and rapidly changing industry that involves numerous risks and uncertainties. The risks and uncertainties described below are not the only ones we face. Other risks and uncertainties, including those that we do not currently consider material, may impair our business. If any of the risks discussed below actually occur, our business, financial condition, operating results or cash flows could be materially adversely affected. This could cause the value of our securities to decline, and you may lose all or part of your investment.

We have marked with an asterisk (*) those risk factors below that include a substantive change from or update to the risk factors included in our Annual Report on Form 10-K, for the year ended December 31, 2014, which was filed with the SEC on March 2, 2015.

Risks Related to Our Business

If we fail to obtain or maintain regulatory approval to commercially market and sell our drugs, or if approval is delayed, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased.

We must obtain and maintain regulatory approval to market and sell our drug products in the U.S. and in jurisdictions outside of the U.S. In the U.S., we must obtain FDA approval for each drug that we intend to commercialize. The FDA approval process is typically lengthy and expensive, and approval is never certain. Products distributed abroad are also subject to government regulation by international regulatory authorities. The approval process in the EU and other countries can also be lengthy and expensive and regulatory approval is also never certain. Naglazyme, Aldurazyme, Kuvan and Vimizim have received regulatory approval to be commercially marketed and sold in the U.S., the EU and other countries. Firdapse has received regulatory approval to be commercially marketed only in the EU.

As part of the recent reauthorization of the Prescription Drug User Fee Act, new biologics are included in a new product review program intended to enhance FDA-sponsor communications to lead to greater first-cycle approval decisions. As part of this program, applications for new biologics are subject to either a 12-month standard or 8-month priority review period that begins from the date of application submission. However, since this is a new product review program and few products have completed this new review process, the priority review period may take longer than eight months and the standard review period may take longer than 12 months. Similarly, although the EMA has an accelerated approval process, the timelines mandated by the regulations are subject to the possibility of substantial delays.

In addition, the FDA and its international equivalents have substantial discretion over the approval process for pharmaceutical products. As such, these regulatory agencies may in the end not agree that we have demonstrated the requisite level of product safety and efficacy to grant approval and may require additional data. If we fail to obtain regulatory approval for our product candidates, we will be unable to market and sell those drug products. Because of the risks and uncertainties in pharmaceutical development, our product candidates could take a significantly longer time to gain regulatory approval than we expect or may never gain approval. We also rely on independent third-party contract research organizations (CROs) to file some of our ex-U.S. and ex-EU marketing applications and important aspects of the services performed for us by the CROs are out of our direct control. If we fail to adequately manage our CROs, if the CRO elects to prioritize work on our projects below other projects or if there is any dispute or disruption in our relationship with our CROs, the filing of our applications may be delayed.

From time to time during the regulatory approval process for our products and our product candidates, we engage in discussions with the FDA and comparable international regulatory authorities regarding the regulatory requirements for our development programs. To the extent appropriate, we accommodate the requests of the regulatory authorities and, to date, we have generally been able to reach reasonable accommodations and resolutions regarding the underlying issues. However, we are often unable to determine the outcome of such deliberations until they are final. If we are unable to effectively and efficiently resolve and comply with the inquiries and requests of the FDA and other non-U.S. regulatory authorities, the approval of our product candidates may be delayed and their value may be reduced.

After any of our products receive regulatory approval, they remain subject to ongoing regulation, which can impact, among other things product labeling, manufacturing practices, adverse event reporting, storage, expiration, distribution, advertising and promotion, record keeping and import and export. If we do not comply with the

applicable regulations, the range of possible sanctions includes issuance of warning or untitled letters or adverse publicity, product recalls or seizures, fines, total or partial suspensions of production and/or distribution, suspension of marketing applications, and other enforcement actions, including injunctions and civil or criminal prosecution. The FDA and comparable international regulatory agencies can withdraw a product's approval under some circumstances, such as the failure to comply with regulatory requirements or unexpected safety issues. Further, the FDA often requires post-marketing testing and surveillance to monitor the effects of approved products. The FDA and comparable international regulatory agencies may condition approval of our product candidates on the completion of such post-marketing clinical studies. These post-marketing studies may suggest that a product causes undesirable side effects or may present a risk to the patient. If data we collect from post-marketing studies suggest that one of our approved products may present a risk to safety, the government authorities could withdraw our product approval, suspend production or place other marketing restrictions on our products. If regulatory sanctions are applied or if regulatory approval is delayed or withdrawn, the value of our company and our operating results will be adversely affected. Additionally, we will be unable to generate revenue from the sale of these products, our potential for generating positive cash flow will be diminished and the capital necessary to fund our operations will be increased.

If we fail to obtain or maintain orphan drug exclusivity for some of our products, our competitors may sell products to treat the same conditions and our revenues will be reduced.

As part of our business strategy, we intend to develop some drugs that may be eligible for FDA and EU orphan drug designation. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the U.S. The company that first obtains FDA approval for a designated orphan drug for a given rare disease receives marketing exclusivity for use of that drug for the stated condition for a period of seven years. Orphan drug exclusive marketing rights may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug. Similar regulations are available in the EU with a ten-year period of market exclusivity.

Because the extent and scope of patent protection for some of our drug products is limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible drugs, we plan to rely on the exclusivity period under the Orphan Drug Act to maintain a competitive position. If we do not obtain orphan drug exclusivity for our drug products that do not have broad patent protection, our competitors may then sell the same drug to treat the same condition and our revenues will be reduced.

Even though we have obtained orphan drug designation for certain of our products and product candidates and even if we obtain orphan drug designation for our future product candidates, due to the uncertainties associated with developing biopharmaceutical products, we may not be the first to obtain marketing approval for any particular orphan indication, which means that we may not obtain orphan drug exclusivity and could also potentially be blocked from approval until the first product's orphan drug exclusivity period expires. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved and granted orphan drug exclusivity, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process.

We may face competition from biological products approved through an abbreviated regulatory pathway.

Our Naglazyme, Aldurazyme and Vimizim products are regulated by the FDA as biologics under the Federal Food, Drug, and Cosmetic Act (the FDC Act) and the Public Health Service Act (the PHS Act). Biologics require the submission of a biologics license application (BLA) and approval by the FDA prior to being marketed in the U.S. Historically, a biologic product approved under a BLA was not subject to the generic drug review and approval provisions of the FDC Act. However, the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (as amended, the PPACA) created a regulatory pathway under the PHS Act for the abbreviated approval for biological products that are demonstrated to be "biosimilar" or "interchangeable" with an FDA-approved biological product. In order to meet the standard of interchangeability, a sponsor must demonstrate that the biosimilar product can be expected to produce the same clinical result as the reference product, and for a product that is administered more than once, that the risk of switching between the reference product and biosimilar product is not greater than the risk of maintaining the patient on the reference product. Such biosimilars would reference biological products approved in the U.S. The law establishes a period of 12 years of data exclusivity for reference products, which protects the data in the original BLA by prohibiting sponsors of biosimilars from gaining FDA approval based in part on reference to data in the original BLA. Our products approved under BLAs, as well as products in development that may be approved under BLAs, could be reference products for such biosimilar marketing applications.

42

To obtain regulatory approval to market our products, preclinical studies and costly and lengthy preclinical and clinical trials are required and the results of the studies and trials are highly uncertain.

As part of the regulatory approval process we must conduct, at our own expense, preclinical studies in the laboratory and clinical trials on humans for each product candidate. We expect the number of preclinical studies and clinical trials that the regulatory authorities will require will vary depending on the product candidate, the disease or condition the drug is being developed to address and regulations applicable to the particular drug. Generally, the number and size of clinical trials required for approval increase based on the expected patient population that may be treated with a drug. We may need to perform multiple preclinical studies using various doses and formulations before we can begin clinical trials, which could result in delays in our ability to market any of our product candidates. Furthermore, even if we obtain favorable results in preclinical studies, the results in humans may be significantly different. After we have conducted preclinical studies, we must demonstrate that our drug products are safe and efficacious for use in the targeted human patients in order to receive regulatory approval for commercial sale. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials, and favorable data from interim analyses do not ensure the final results of a trial will be favorable. Product candidates may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical trials, or despite having favorable data in connection with an interim analysis. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Our future clinical trial results may not be successful.

Adverse or inconclusive clinical results would stop us from filing for regulatory approval of our product candidates. Additional factors that can cause delay or termination of our clinical trials include:

- slow or insufficient patient enrollment;
- slow recruitment of, and completion of necessary institutional approvals at, clinical sites;
- longer treatment time required to demonstrate efficacy;
- lack of sufficient supplies of the product candidate;
- adverse medical events or side effects in treated patients;
- lack of effectiveness of the product candidate being tested; and
- regulatory requests for additional clinical trials or pre-clinical studies.

Typically, if a drug product is intended to treat a chronic disease, as is the case with some of our product candidates, safety and efficacy data must be gathered over an extended period of time, which can range from nine months to three years or more. We also rely on independent third-party CROs to perform most of our clinical studies and many important aspects of the services performed for us by the CROs are out of our direct control. If we fail to adequately manage our CROs, or if there is any dispute or disruption in our relationship with our CROs, our clinical trials may be delayed. Moreover, in our regulatory submissions, we rely on the quality and validity of the clinical work performed by third-party CROs. If any of our CROs' processes, methodologies or results were determined to be not conducted in accordance with current good clinical practices, invalid or inadequate, our own clinical data and results and related regulatory approvals could adversely be impacted.

If we continue to incur operating losses for a period longer than anticipated, we may be unable to continue our operations at planned levels and be forced to reduce our operations.

Since we began operations in March 1997, we have been engaged in substantial research and development and operated at a net loss until 2008. Although we were profitable in 2008, 2010 and the third quarter of 2014, we operated at a net loss in 2009, 2011, 2012, 2013 and 2014. Based upon our current plan for investments in research and development for existing and new programs, we expect to operate at a net loss for at least the next 12 months. Our

future profitability depends on our marketing and selling of Vimizim, Naglazyme, Kuvan and Firdapse, the successful continued commercialization of Aldurazyme by Genzyme, the receipt of regulatory approval of our product candidates, our ability to successfully manufacture and market any approved drugs, either by ourselves or jointly with others, our spending on our development programs and the impact of any possible future business development transactions. The extent of our future losses and the timing of profitability are highly uncertain. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce our operations.

If we fail to comply with manufacturing regulations, our financial results and financial condition will be adversely affected.

Before we can begin commercial manufacture of our products, regulatory authorities must approve marketing applications that identify manufacturing facilities operated by us or our contract manufacturers that have passed regulatory inspection and manufacturing processes that are acceptable to the regulatory authorities. In addition, our pharmaceutical manufacturing facilities are continuously subject to inspection by the FDA and international regulatory authorities, before and after product approval. Our manufacturing facilities in the U.S. have been approved by the FDA, the European Commission (the EC), and health agencies in other countries for the manufacture of Aldurazyme and Naglazyme. In addition, our third-party manufacturers' facilities involved with the manufacture of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse have also been inspected and approved by various regulatory authorities. Although we are not involved in the day-to-day operations of our contract manufacturers, we are ultimately responsible for ensuring that our products are manufactured in accordance with current Good Manufacturing Practices (cGMP) regulations. The manufacturing facility located in Shanbally, Cork, Ireland that we purchased in 2011 has not yet been approved by the FDA or the EMA to manufacture any of our products. We intend to make a substantial investment in the build-out of the Shanbally facility in order to manufacture Vimizim and other products. If the facility is not ultimately approved by the FDA or the EMA to manufacture any of our products, we will not be able to manufacture Vimizim or other products at this facility and we may not be able to meet the anticipated commercial demand for Vimizim which would have an adverse effect on our financial results.

Due to the complexity of the processes used to manufacture our products and product candidates, we may be unable to continue to pass or initially pass federal or international regulatory inspections in a cost-effective manner. For the same reason, any potential third-party manufacturer of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse or our product candidates may be unable to comply with cGMP regulations in a cost-effective manner and may be unable to initially or continue to pass a federal or international regulatory inspection.

If we, or third-party manufacturers with whom we contract, are unable to comply with manufacturing regulations, we may be subject to delay of approval of our product candidates, warning or untitled letters, fines, unanticipated compliance expenses, recall or seizure of our products, total or partial suspension of production and/or enforcement actions, including injunctions, and criminal or civil prosecution. These possible sanctions would adversely affect our financial results and financial condition.

*If we fail to obtain the capital necessary to fund our operations, our financial results and financial condition will be adversely affected and we will have to delay or terminate some or all of our product development programs.

As of March 31, 2015, we had cash, cash equivalents and short and long-term investments totaling $1,232.6 million and long-term debt obligations of $781.9 million (undiscounted). In January 2015, we paid $620.7 million for 34,970,514 Prosensa Shares, representing approximately 96.8% of all outstanding Prosensa Shares, and $38.6 million for the options that vested pursuant to the definitive purchase agreement. In February 2015, we completed the Prosensa asset transfer and paid $20.8 million to the remaining Prosensa shareholders. We (through our indirect wholly-owned subsidiaries) funded the acquisition with our available cash balances. We expect to pay up to $160.0 million if certain development milestones are attained. In October 2013, we completed an offering of senior subordinated convertible notes and received net proceeds of approximately $696.4 million, after deducting commissions, estimated offering expenses payable by us and the purchase of the related capped calls. We will need cash to not only repay the principal amount of our 0.75% senior subordinated convertible notes due 2018 and 1.50% senior subordinated convertible notes due in 2020 (collectively, the Notes) but also the ongoing interest due on the Notes during their term. In March 2014, we completed an offering of 1,500,000 shares of our common stock at a price of $78.45 per share and received net proceeds of $117.5 million. In January 2015, we completed an offering of 9,775,000 shares of our common stock at a price of $93.25 per share and received net proceeds of approximately

$888.3 million. We may require additional financing to fund our future operations, including the commercialization of our approved drugs and drug product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. We may be unable to raise additional financing, if needed, due to a variety of factors, including our financial condition, the status of our product programs, and the general condition of the financial markets. If we fail to raise any necessary additional financing we may have to delay or terminate some or all of our product development programs and our financial condition and operating results will be adversely affected.

We expect to continue to spend substantial amounts of capital for our operations for the foreseeable future. The amount of capital we will need depends on many factors, including:

- our ability to successfully market and sell Vimizim, Naglazyme, Kuvan and Firdapse;

- Genzyme's ability to continue to successfully commercialize Aldurazyme;

- the progress and success of our preclinical studies and clinical trials (including studies and the manufacture of materials);

- the timing, number, size and scope of our preclinical studies and clinical trials;

- the time and cost necessary to obtain regulatory approvals and the costs of post-marketing studies which may be required by regulatory authorities;

- the time and cost necessary to develop commercial manufacturing processes, including quality systems, and to build or acquire manufacturing capabilities;

- the progress of research programs carried out by us;

- our possible achievement of milestones identified in our purchase agreements with the former stockholders of LEAD Therapeutics, Inc., ZyStor, Huxley Pharmaceuticals, Inc., and Zacharon Pharmaceuticals Inc. that trigger related milestone payments;

- any changes made to, or new developments in, our existing collaborative, licensing and other commercial relationships or any new collaborative, licensing and other commercial relationships that we may establish; and

- whether our convertible debt is converted to common stock in the future.

Moreover, our fixed expenses such as rent, license payments, interest expense and other contractual commitments are substantial and may increase in the future. These fixed expenses may increase because we may enter into:

- additional licenses and collaborative agreements;

- additional contracts for product manufacturing; and

- additional financing facilities.

We may need to raise additional funds from equity or debt securities, loans or collaborative agreements if we are unable to satisfy our liquidity requirements. The sale of additional securities may result in additional dilution to our stockholders. Furthermore, additional financing may not be available in amounts or on terms satisfactory to us or at all. This could result in the delay, reduction or termination of our research, which could harm our business.

If we are unable to successfully develop and maintain manufacturing processes for our drug products to produce sufficient quantities at acceptable costs, we may be unable to meet demand for our products and lose potential revenue, have reduced margins or be forced to terminate a program.

Due to the complexity of manufacturing our products, we may not be able to manufacture drug products successfully with a commercially viable process or at a scale large enough to support their respective commercial markets or at

acceptable margins.

The development of commercially viable manufacturing processes typically is very difficult to achieve and is often very expensive and may require extended periods of time. Changes in manufacturing processes (including manufacturing cell lines), equipment or facilities may require us to complete clinical trials to receive regulatory approval of any manufacturing improvements. Also, we may be required to demonstrate product comparability between a biological product made after a manufacturing change and the product made before implementation of the change through additional types of analytical and functional testing or may have to complete additional clinical studies. If we contract for manufacturing services with an unproven process, our contractor is subject to the same uncertainties, high standards and regulatory controls, and may therefore experience difficulty if further process development is necessary.

Even a developed manufacturing process can encounter difficulties. Problems may arise during manufacturing for a variety of reasons, including human error, mechanical breakdowns, problems with raw materials and cell banks, malfunctions of internal information technology systems, and other events that cannot always be prevented or anticipated. Many of the processes include biological systems, which add significant complexity, as compared to chemical synthesis. We expect that, from time to time, consistent with biotechnology industry expectations, certain production lots will fail to produce product that meets our quality control release acceptance criteria. To date, our historical failure rates for all of our product programs, including Naglazyme, Aldurazyme and Vimizim, have been within our expectations, which are based on industry norms. If the failure rate increased substantially, we could experience increased costs, lost revenue, damage to customer relations, time and expense investigating the cause and, depending upon the cause, similar losses with respect to other lots or products. If problems are not discovered before the product is released to the market, recall and product liability costs may also be incurred.

In order to produce product within our time and cost parameters, we must continue to produce product within our expected success rate and yield expectations. Because of the complexity of our manufacturing processes, it may be difficult or impossible for us to determine the cause of any particular lot failure and we must effectively take corrective action in response to any failure in a timely manner.

Although we have entered into contractual relationships with third-party manufacturers to produce the active ingredient in Kuvan and Firdapse, if those manufacturers are unwilling or unable to fulfill their contractual obligations, we may be unable to meet demand for these products or sell these products at all and we may lose potential revenue. We have contracts for the production of final product for Kuvan and Firdapse. We also rely on third-parties for portions of the manufacture of Naglazyme and Aldurazyme. If those manufacturers are unwilling or unable to fulfill their contractual obligations or satisfy demand outside of or in excess of the contractual obligations, we may be unable to meet demand for these products or sell these products at all and we may lose potential revenue. Further, the availability of suitable contract manufacturing capacity at scheduled or optimum times is not certain.

In addition, our manufacturing processes subject us to a variety of federal, state and local laws and regulations governing the use, generation, manufacture, storage, handling and disposal of hazardous materials and wastes resulting from their use. We may incur significant costs in complying with these laws and regulations.

If we are unable to effectively address manufacturing issues, we may be unable to meet demand for our products and lose potential revenue, have reduced margins, or be forced to terminate a program.

Our manufacturing facility for Naglazyme, Aldurazyme and Vimizim is located near known earthquake fault zones, and the occurrence of an earthquake or other catastrophic disaster could cause damage to our facility and equipment, or that of our third-party manufacturers or single-source suppliers, which could materially impair our ability to manufacture Naglazyme, Aldurazyme and Vimizim or our third-party manufacturer’s ability to manufacture Kuvan or Firdapse.

Our Galli Drive facility located in Novato, California is currently our only manufacturing facility for Naglazyme, Aldurazyme and Vimizim. It is located in the San Francisco Bay Area near known earthquake fault zones and is vulnerable to significant damage from earthquakes. We, the third-party manufacturers with whom we contract and our single-source suppliers of raw materials, which include many of our critical raw materials, are also vulnerable to damage from other types of disasters, including fires, floods, power loss and similar events. If any disaster were to occur, or any terrorist or criminal activity caused significant damage to our facilities or the facilities of our third-party manufacturers and suppliers, our ability to manufacture Naglazyme, Aldurazyme and Vimizim, or to have Kuvan or Firdapse manufactured, could be seriously, or potentially completely impaired, and our commercialization efforts and revenue could be seriously impaired. The insurance that we carry, the inventory that we maintain and our risk mitigation plans may not be adequate to cover our losses resulting from disasters or other business interruptions.

Supply interruptions may disrupt our inventory levels and the availability of our products and product candidates and cause delays in obtaining regulatory approval for our product candidates, or harm our business by reducing our revenues.

Numerous factors could cause interruptions in the supply of our products and product candidates, including:

- timing, scheduling and prioritization of production by our contract manufacturers or a breach of our agreements by our contract manufacturers;

- labor interruptions;

- changes in our sources for manufacturing;

·the timing and delivery of shipments;

·our failure to locate and obtain replacement manufacturers as needed on a timely basis; and

·conditions affecting the cost and availability of raw materials.

Any interruption in the supply of finished products could hinder our ability to distribute finished products to meet commercial demand.

With respect to our product candidates, production of product is necessary to perform clinical trials and successful registration batches are necessary to file for approval to commercially market and sell product candidates. Delays in obtaining clinical material or registration batches could adversely impact our clinical trials and delay regulatory approval for our product candidates.

Because the target patient populations for our products are small, we must achieve significant market share and maintain high per-patient prices for our products to achieve profitability.

All of our products target diseases with small patient populations. As a result, our per-patient prices must be relatively high in order to recover our development and manufacturing costs and achieve profitability. For Naglazyme and Vimizim we must market worldwide to achieve significant market penetration of the product. In addition, because the number of potential patients in the disease populations are small, it is not only important to find patients who begin therapy to achieve significant market penetration of the product, but we also need to be able to maintain these patients on therapy for an extended period of time. Due to the expected costs of treatment for our products for genetic diseases, we may be unable to maintain or obtain sufficient market share at a price high enough to justify our product development efforts and manufacturing expenses.

If we fail to obtain an adequate level of coverage and reimbursement for our drug products by third-party payers, the sales of our drugs would be adversely affected or there may be no commercially viable markets for our products.

The course of treatment for patients using our products is expensive. We expect patients to need treatment for extended periods, and for some products throughout the lifetimes of the patients. We expect that most families of patients will not be capable of paying for this treatment themselves. There will be no commercially viable market for our products without coverage and reimbursement from third-party payers. Additionally, even if there is a commercially viable market, if the level of reimbursement is below our expectations, our revenue and gross margins will be adversely affected.

Third-party payers, such as government or private health care insurers, carefully review and increasingly challenge the prices charged for drugs. Reimbursement rates from private companies vary depending on the third-party payer, the insurance plan and other factors. Reimbursement systems in international markets vary significantly by country and by region, and reimbursement approvals must be obtained on a country-by-country basis.

Reimbursement in the EU and many other territories must be negotiated on a country-by-country basis and in many countries the product cannot be commercially launched until reimbursement is approved. The timing to complete the negotiation process in each country is highly uncertain, and in some countries we expect that it may exceed 12 months. Even after a price is negotiated, countries frequently request or require adjustments to the price and other concessions over time.

For our future products, we will not know what the reimbursement rates will be until we are ready to market the product and we actually negotiate the rates. If we are unable to obtain sufficiently high reimbursement rates for our products, they may not be commercially viable or our future revenues and gross margins may be adversely affected.

A significant portion of our international sales are made based on special access programs, and changes to these programs could adversely affect our product sales and revenue in these countries.

We make a significant portion of our international sales of Naglazyme through special access or “named patient” programs, which do not require full product approval. We expect to also utilize these programs for Vimizim. The specifics of the programs vary from country to country. Generally, special approval must be obtained for each patient. The approval normally requires an application or a lawsuit accompanied by evidence of medical need. Generally, the approvals for each patient must be renewed from time to time.

These programs are not well defined in some countries and are subject to changes in requirements and funding levels. Any change to these programs could adversely affect our ability to sell our products in those countries and delay sales. If the programs are not funded by the respective government, there could be insufficient funds to pay for all patients. Further, governments have in the past

undertaken and may in the future undertake, unofficial measures to limit purchases of our products, including initially denying coverage for purchasers, delaying orders and denying or taking excessively long to approve customs clearance. Any such actions could materially delay or reduce our revenues from such countries.

Without the special access programs, we would need to seek full product approval to commercially market and sell our products. This can be an expensive and time-consuming process and may subject our products to additional price controls. Because the number of patients is so small in some countries, it may not be economically feasible to seek and maintain a full product approval, and therefore the sales in such country would be permanently reduced or eliminated. For all of these reasons, if the special access programs that we are currently using are eliminated or restricted, our revenues could be adversely affected.

If we fail to compete successfully with respect to product sales, we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product and our revenue could be adversely affected.

Our competitors may develop, manufacture and market products that are more effective or less expensive than ours. They may also obtain regulatory approvals for their products faster than we can obtain them (including those products with orphan drug designation) or commercialize their products before we do. If we do not compete successfully, our revenue would be adversely affected, and we may be unable to generate sufficient sales to recover our expenses related to the development of a product program or to justify continued marketing of a product.

Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenue and results of operations.

We expect that coverage and reimbursement may be increasingly restricted both in the U.S. and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. Governmental and private third-party payers have proposed health care reforms and cost reductions. A number of federal and state proposals to control the cost of health care, including the cost of drug treatments, have been made in the U.S. In some international markets, the government controls the pricing, which can affect the profitability of drugs. Current government regulations and possible future legislation regarding health care may affect coverage and reimbursement for medical treatment by third-party payers, which may render our products not commercially viable or may adversely affect our future revenues and gross margins.

International operations are also generally subject to extensive price and market regulations, and there are many proposals for additional cost-containment measures, including proposals that would directly or indirectly impose additional price controls or mandatory price cuts or reduce the value of our intellectual property portfolio. As part of these cost containment measures, some countries have imposed or threatened to impose revenue caps limiting the annual volume of sales of Naglazyme. To the extent that these caps are significantly below actual demand, our future revenues and gross margins may be adversely affected.

We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our current and future products, which would adversely affect our revenue and results of operations.

Government health care reform could increase our costs, and would adversely affect our revenue and results of operations.

Our industry is highly regulated and changes in law may adversely impact our business, operations or financial results. The PPACA is a sweeping measure intended to expand healthcare coverage within the U.S., primarily through the imposition of health insurance mandates on employers and individuals and expansion of the Medicaid program.

Several provisions of the law, which have varying effective dates, may affect us and will likely increase certain of our costs. For example, the Medicaid rebate rate was increased and the volume of rebated drugs has been expanded to include beneficiaries in Medicaid managed care organizations. Among other things, the PPACA also expanded the 340B drug discount program (excluding orphan drugs), including the creation of new penalties for non-compliance; included a 50% discount on brand name drugs for Medicare Part D participants in the coverage gap, or “donut hole,” and imposed a new fee on certain manufacturers and importers of branded prescription drugs (excluding orphan drugs under certain conditions). The law also revised the definition of “average manufacturer price” for reporting purposes, which could increase the amount of the Medicaid drug rebates paid to states.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. These changes include aggregate reductions in Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013 and will remain in effect through 2024 unless additional Congressional action is taken. In January 2013, President Obama signed into law the

American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several types of providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on our customers and, accordingly, our financial operations.

We anticipate that the PPACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and an additional downward pressure on the reimbursement our customers may receive for our products. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payers. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability or commercialize our products.

We face credit risks from customers outside of the U.S. that may adversely affect our results of operations.

Our product sales to government-owned or supported customers in various countries outside of the U.S. are subject to significant payment delays due to government funding and reimbursement practices. This has resulted and may continue to result in an increase in days sales outstanding due to the average length of time that we have accounts receivable outstanding. If significant changes were to occur in the reimbursement practices of these governments or if government funding becomes unavailable, we may not be able to collect on amounts due to us from these customers and our results of operations would be adversely affected.

If we are found in violation of federal or state “fraud and abuse” laws, we may be required to pay a penalty or be suspended from participation in federal or state health care programs, which may adversely affect our business, financial condition and results of operation.

We are subject to various federal and state health care fraud and abuse laws, including anti-kickback laws, false claims laws and laws related to ensuring compliance. The federal health care program anti-kickback statute makes it illegal for any person, including a pharmaceutical company, to knowingly and willfully offer, solicit, pay or receive any remuneration, directly or indirectly, in exchange for or to induce the referral of business, including the purchase, order or prescription of a particular drug, for which payment may be made under federal health care programs, such as Medicare and Medicaid. Under federal government regulations, certain arrangements, or safe harbors, are deemed not to violate the federal anti-kickback statute. However, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration not intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exemption or safe harbor. Our practices may not in all cases meet all of the criteria for safe harbor protection from anti-kickback liability, although we seek to comply with these safe harbors. Violations of the anti-kickback statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs.

Federal and state false claims laws prohibit any person from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. Under the Health Insurance Portability and Accountability Act of 1996, we also are prohibited from knowingly and willfully executing a scheme to defraud any health care benefit program, including private payers, or knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for health care benefits, items or services. Sanctions under these federal and state laws may include civil monetary penalties, exclusion of a manufacturer’s products from reimbursement under government programs, criminal fines and imprisonment.

Many states have adopted laws similar to the federal anti-kickback statute, some of which apply to referral of patients for health care services reimbursed by any source, not just governmental payers.

Substantial new provisions affecting compliance have also been adopted, which may require us to modify our business practices with health care practitioners. The PPACA, among other things, requires drug manufacturers to collect and report information on payments or transfers of value to physicians and teaching hospitals, as well as investment and ownership interests held by physicians and their immediate family members during the preceding calendar year. Failure to submit required information may result in civil monetary penalties. Manufacturers were required to begin collecting required information on August 1, 2013 and the Centers for Medicare & Medicaid Services (CMS) made public the reported data in a searchable form on September 30, 2014. Manufacturers are required to submit reports to CMS by the 90th day of each subsequent calendar year.

In addition, there has been a recent trend of increased state regulation of payments made to physicians. Certain states mandate implementation of compliance programs, compliance with the Office of Inspector General Compliance Program Guidance for Pharmaceutical Manufacturers and the PhRMA Code on Interactions with Healthcare Professionals, and/or the tracking and reporting

of gifts, compensation and other remuneration to physicians. The shifting compliance environment and the need to implement systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that a pharmaceutical manufacturer may violate one or more of the requirements.

While we believe we have structured our business arrangements to comply with these laws, because of the breadth of these laws, the narrowness of available statutory and regulatory exceptions and the increased focus by law enforcement agencies in enforcing such laws, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent health care reform legislation has strengthened, these laws. For example, the PPACA, among other things, amends the intent requirement of the federal anti-kickback and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. Moreover, the PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the False Claims Act. If we are found in violation of one of these laws, we may be subject to criminal, civil or administrative sanctions, including debarment, suspension or exclusion from participation in federal or state health care programs any of which could adversely affect our business, financial condition and results of operation.

We conduct a significant amount of our sales and operations outside of the U.S., which subjects us to additional business risks that could adversely affect our revenue and results of operations.

A significant portion of the sales of Aldurazyme and Naglazyme and all of the sales of Firdapse are generated from countries other than the U.S. Additionally, we have operations in several European countries, Brazil, other Latin American countries, Turkey and other Asian countries. We expect that we will continue to expand our international operations in the future. International operations inherently subject us to a number of risks and uncertainties, including:

- changes in international regulatory and compliance requirements that could restrict our ability to manufacture, market and sell our products;
- political and economic instability;
- diminished protection of intellectual property in some countries outside of the U.S.;
- trade protection measures and import or export licensing requirements;
- difficulty in staffing and managing international operations;
- differing labor regulations and business practices;
- potentially negative consequences from changes in or interpretations of tax laws;
- changes in international medical reimbursement policies and programs;
- financial risks such as longer payment cycles, difficulty collecting accounts receivable and exposure to fluctuations in foreign currency exchange rates; and
- regulatory and compliance risks that relate to maintaining accurate information and control over sales and distributors' and service providers' activities that may fall within the purview of the Foreign Corrupt Practices Act (the FCPA).

Any of these factors may, individually or as a group, have a material adverse effect on our business and results of operations.

As we continue to expand our existing international operations, we may encounter new risks. For example, as we focus on building our international sales and distribution networks in new geographic regions, we must continue to develop relationships with qualified local distributors and trading companies. If we are not successful in developing and maintaining these relationships, we may not be able to grow sales in these geographic regions. These or other similar risks could adversely affect our revenue and profitability.

Our international operations pose currency risks, which may adversely affect our operating results and net income.

A significant and growing portion of our revenues and earnings, as well as our substantial international net assets, are exposed to changes in foreign exchange rates. As we operate in multiple foreign currencies, including the euro, the Brazilian real, the U.K. pound, the Canadian dollar, the Swiss Franc, the Japanese yen and several other currencies, changes in those currencies relative to the U.S. dollar will impact our revenues and expenses. If the U.S. dollar were to weaken against another currency, assuming all other variables remained constant, our revenues would increase, having a positive impact on earnings, and our overall expenses would increase, having a negative impact on earnings. Conversely, if the U.S. dollar were to strengthen against another currency, assuming all other variables remained constant, our revenues would decrease, having a negative impact on earnings, and our overall expenses would

decrease, having a positive impact on earnings. In addition, because our financial statements are reported in U.S. dollars, changes in currency exchange rates between the U.S. dollar and other currencies have had, and will continue to have, an impact on our results of operations. Therefore, significant changes in foreign exchange rates can impact our results and our financial guidance.

From time to time, we may implement currency hedges intended to reduce our exposure to changes in foreign currency exchange rates. However, our hedging strategies may not be successful, and any of our unhedged foreign exchange exposures will continue to be subject to market fluctuations. Moreover, when we do implement currency hedges, we only hedge our net exposure, or the difference between our revenues in a currency and the offsetting expenses in that currency. Since we do not generally hedge the portion of our revenues that has offsetting expenses in that currency, our revenues can be particularly affected by changes in exchange rates. These risks could cause a material adverse effect on our business, financial position and results of operations and could cause the market value of our common stock to decline.

If we are unable to protect our proprietary technology, we may not be able to compete as effectively.

Where appropriate, we seek patent protection for certain aspects of our technology. Patent protection may not be available for some of the products we are developing. If we must spend significant time and money protecting or enforcing our patents, designing around patents held by others or licensing, potentially for large fees, patents or other proprietary rights held by others, our business and financial prospects may be harmed.

The patent positions of biopharmaceutical products are complex and uncertain. The scope and extent of patent protection for some of our products and product candidates are particularly uncertain because key information on some of our product candidates has existed in the public domain for many years. The composition and genetic sequences of animal and/or human versions of Naglazyme, Aldurazyme and many of our product candidates have been published and are believed to be in the public domain. The chemical structure of 6R-BH4 (the active ingredient in Kuvan) and 3,4-DAP (the active ingredient in Firdapse) have also been published. Publication of this information may prevent us from obtaining or enforcing patents relating to our products and product candidates, including without limitation composition-of-matter patents, which are generally believed to offer the strongest patent protection.

We own or have licensed patents and patent applications related to Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse. However, these patents and patent applications do not ensure the protection of our intellectual property for a number of reasons, including without limitation the following:

- With respect to pending patent applications, unless and until actually issued, the protective value of these applications is impossible to determine. We do not know whether our patent applications will result in issued patents.

- Competitors may interfere with our patent process in a variety of ways. Competitors may claim that they invented the claimed invention prior to us or that they filed their application for a patent on a claimed invention before we did. Competitors may also claim that we are infringing on their patents and therefore we cannot practice our technology. Competitors may also contest our patents by showing the patent examiner or a court that the invention was not original, was not novel or was obvious, for example. In litigation, a competitor could claim that our issued patents are not valid or are unenforceable for a number of reasons. If a court agrees, we would not be able to enforce that patent. We have no meaningful experience with competitors interfering with or challenging the validity or enforceability of our patents or patent applications.

.

Enforcing patents is expensive and may absorb significant time of our management. Management would spend less time and resources on developing products, which could increase our operating expenses and delay product programs. We may not have the financial ability to sustain a patent infringement action, or it may not be financially reasonable to do so.

- Receipt of a patent may not provide much, if any, practical protection. For example, if we receive a patent with a narrow scope, then it will be easier for competitors to design products that do not infringe on our patent

- The Leahy-Smith America Invents Act of 2011, which reformed certain patent laws in the U.S., may create additional uncertainty. Among the significant changes are switching from a “first-to-invent” system to a “first-to-file” system, and the implementation of new procedures that permit competitors to challenge our patents in the U.S. Patent and Trademark Office after grant.

It is also unclear whether our trade secrets are adequately protected. Our employees, consultants or contractors may unintentionally or willfully disclose trade secrets to competitors. Enforcing a claim that someone else illegally obtained and is using our trade secrets, as with patent litigation, is expensive and time consuming, requires significant resources and the outcome is unpredictable. In addition, courts outside of the U.S. are sometimes less willing to protect trade secrets. Furthermore, our competitors may independently develop equivalent knowledge, methods and know-how, in which case we would not be able to enforce our trade secret rights against such competitors.

If we are unable to protect our intellectual property, third parties could develop competing products, which could adversely affect our revenue and financial results generally.

Competitors and other third parties may have developed intellectual property that could limit our ability to market and commercialize our products and product candidates, if approved.

Similar to us, competitors continually seek intellectual property protection for their technology. Several of our development programs, such as talazoparib, reveglucosidase alfa, BMN 111 and BMN 270, focus on therapeutic areas that have been the subject of extensive research and development by third parties for many years. Due to the amount of intellectual property in our field of technology, we cannot be certain that we do not infringe intellectual property rights of competitors or that we will not infringe intellectual property rights of competitors granted or created in the future. For example, if a patent holder believes our product infringes its patent, the patent holder may sue us even if we have received patent protection for our technology. If someone else claims we infringe its intellectual property, we would face a number of issues, including the following:

- Defending a lawsuit takes significant executive resources and can be very expensive.

- If a court decides that our product infringes a competitor’s intellectual property, we may have to pay substantial damages.

- With respect to patents, in addition to requiring us to pay substantial damages, a court may prohibit us from making, selling, offering to sell, importing or using our product unless the patent holder licenses the patent to us. The patent holder is not required to grant us a license. If a license is available, it may not be available on commercially reasonable terms. For example, we may have to pay substantial royalties or grant cross licenses to our patents and patent applications.

- We may need to redesign our product so it does not infringe the intellectual property rights of others.

- Redesigning our product so it does not infringe the intellectual property rights of competitors may not be possible or could require substantial funds and time.

We may also support and collaborate in research conducted by government organizations, hospitals, universities or other educational institutions. These research partners may be unwilling to grant us any exclusive rights to technology or products derived from these collaborations.

If we do not obtain required licenses or rights, we could encounter delays in our product development efforts while we attempt to design around other patents or may be prohibited from making, using, importing, offering to sell or selling

products requiring these licenses or rights. There is also a risk that disputes may arise as to the rights to technology or products developed in collaboration with other parties. If we are not able to resolve such disputes and obtain the licenses or rights we need, we may not be able to develop or market our products.

If our Manufacturing, Marketing and Sales Agreement with Genzyme were terminated, we could be prevented from continuing to commercialize Aldurazyme or our ability to successfully commercialize Aldurazyme would be delayed or diminished.

Either party may terminate the Manufacturing, Marketing and Sales Agreement (the MMS Agreement) between Genzyme and us related to Aldurazyme for specified reasons, including if the other party is in material breach of the MMS Agreement, has experienced a change of control, as such term is defined in the MMS Agreement, or has declared bankruptcy and also is in breach of the MMS Agreement. Although we are not currently in breach of the MMS Agreement, there is a risk that either party could breach the MMS Agreement in the future. Either party may also terminate the MMS Agreement upon one year prior written notice for any reason.

If the MMS Agreement is terminated for breach, the breaching party will transfer its interest in the LLC to the non-breaching party, and the non-breaching party will pay a specified buyout amount for the breaching party's interest in Aldurazyme and in the LLC. If we are the breaching party, we would lose our rights to Aldurazyme and the related intellectual property and regulatory approvals. If the MMS Agreement is terminated without cause, the non-terminating party would have the option, exercisable for one year, to buy out the terminating party's interest in Aldurazyme and in the LLC at a specified buyout amount. If such option is not exercised, all rights to Aldurazyme will be sold and the LLC will be dissolved. In the event of termination of the buyout option without exercise by the non-terminating party as described above, all right and title to Aldurazyme is to be sold to the highest bidder, with the proceeds to be split between Genzyme and us in accordance with our percentage interest in the LLC.

If the MMS Agreement is terminated by either party because the other party declared bankruptcy, the terminating party would be obligated to buy out the other party and would obtain all rights to Aldurazyme exclusively. If the MMS Agreement is terminated by a party because the other party experienced a change of control, the terminating party shall notify the other party, the offeree, of its intent to buy out the offeree's interest in Aldurazyme and the LLC for a stated amount set by the terminating party at its discretion. The offeree must then either accept this offer or agree to buy the terminating party's interest in Aldurazyme and the LLC on those same terms. The party who buys out the other party would then have exclusive worldwide rights to Aldurazyme. The Amended and Restated Collaboration Agreement between us and Genzyme will automatically terminate upon the effective date of the termination of the MMS Agreement and may not be terminated independently from the MMS Agreement.

If we were obligated or given the option to buy out Genzyme's interest in Aldurazyme and the LLC, and thereby gain exclusive rights to Aldurazyme, we may not have sufficient funds to do so and we may not be able to obtain the financing to do so. If we fail to buy out Genzyme's interest, we may be held in breach of the agreement and may lose any claim to the rights to Aldurazyme and the related intellectual property and regulatory approvals. We would then effectively be prohibited from developing and commercializing Aldurazyme. If this happened, not only would our product revenues decrease, but our share price would also decline.

Based on our strategic alliance with Merck Serono, unless Merck Serono "opts in" to the pegvaliase program, we will not realize any cost sharing for the development expenses, development milestones, or royalties for ex-U.S. sales.

In May 2005, we entered into an agreement with Merck Serono for the further development and commercialization of Kuvan (and any other product containing 6R-BH4) and pegvaliase for PKU. Pursuant to that agreement, we received development milestones on Kuvan and receive royalties on sales by Merck Serono. Additionally, we may be entitled to development milestones and royalties related to pegvaliase. However, Merck Serono has "opted out" of the pegvaliase development program. Unless and until it elects to opt in, it is not obligated to pay any of the milestones related to the program or to reimburse us for any of the development costs. Additionally, even though Merck Serono has opted out of the pegvaliase development program, we do not have any right to commercialize pegvaliase outside of the U.S. and Japan or to grant anyone else such rights.

Merck Serono may elect to opt in at any time. If Merck Serono opts in to the pegvaliase development program before the unblinding of the first Phase 3 trial for pegvaliase, it must pay 75% of the Phase 3 costs incurred prior to the opt-in and the $7,000,000 Phase 3 initiation milestone. If it opts in after unblinding of the first Phase 3 trial for pegvaliase, it must pay 100% of the Phase 3 costs incurred prior to the opt-in and the $7,000,000 Phase 3 initiation milestone. Additionally, in all cases after it opts in to the pegvaliase development program, Merck Serono would be obligated to pay one half of future development costs under the agreement and any further milestones due under the agreement. If Merck Serono does not opt in, it will not have the right to use any of the clinical or other independently developed data.

We cannot determine when or if Merck Serono will opt in to the pegvaliase development program. If Merck Serono does not opt in, we will not receive any milestones under the agreement nor will there be any sales outside of the U.S. or Japan generating revenue from royalties or otherwise.

If we fail to compete successfully with respect to acquisitions, joint ventures or other collaboration opportunities, we may be limited in our ability to develop new products and to continue to expand our product pipeline.

Our competitors compete with us to attract organizations for acquisitions, joint ventures, licensing arrangements or other collaborations. To date, several of our product programs have been acquired through acquisitions, such as reveglucosidase alfa and talazoparib and several of our product programs have been developed through licensing or collaborative arrangements, such as Naglazyme, Aldurazyme, Kuvan and Firdapse. These collaborations include licensing proprietary technology from, and other relationships with, academic research institutions. Our future success will depend, in part, on our ability to identify additional opportunities and to successfully enter into partnering or acquisition agreements for those opportunities. If our competitors successfully enter into partnering arrangements or license agreements with academic research institutions, we will then be precluded from pursuing those specific opportunities. Since each of these opportunities is unique, we may not be able to find a substitute. Several pharmaceutical and biotechnology companies have already established themselves in the field of genetic diseases. These companies

have already begun many drug development programs, some of which may target diseases that we are also targeting, and have already entered into partnering and licensing arrangements with academic research institutions, reducing the pool of available opportunities.

Universities and public and private research institutions also compete with us. While these organizations primarily have educational or basic research objectives, they may develop proprietary technology and acquire patents that we may need for the development of our product candidates. We will attempt to license this proprietary technology, if available. These licenses may not be available to us on acceptable terms, if at all. If we are unable to compete successfully with respect to acquisitions, joint venture and other collaboration opportunities, we may be limited in our ability to develop new products and to continue to expand our product pipeline.

*If generic manufacturers use litigation and regulatory means to obtain approval for generic versions of Kuvan, our revenue and results of operations would be adversely affected.

The Drug Price Competition and Patent Term Restoration Act of 1984, known as the Hatch-Waxman Act, permits the FDA to approve ANDAs for generic versions of branded drugs. We refer to this process as the ANDA process. The ANDA process permits competitor companies to obtain marketing approval for a drug with the same active ingredient as a branded drug, but does not generally require the conduct and submission of clinical efficacy studies for the generic product. In place of such clinical studies, an ANDA applicant usually needs only to submit data demonstrating that its product is bioequivalent to the branded product. Pursuant to the Hatch-Waxman Act, companies were permitted to file ANDA applications for proposed generic versions of Kuvan (sapropterin hydrochloride) at any time after December 2011.

BioMarin owns several patents that cover Kuvan (sapropterin dihydrochloride), and we have listed those patents in conjunction with that product in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). The Hatch-Waxman Act requires an ANDA applicant seeking FDA approval of its proposed generic product prior to the expiration of our Orange Book-listed patents to certify that the applicant believes that our patents are invalid or will not be infringed by the manufacture, use or sale of the drug for which the application has been submitted (a paragraph IV certification) and notify us of such certification (a paragraph IV notice). Upon receipt of a paragraph IV notice, the Hatch-Waxman Act allows us, with proper basis, to bring an action for patent infringement against the ANDA filer, asking that the proposed generic product not be approved until after our patents expire. If we commence a lawsuit within 45 days from receipt of the paragraph IV notice, the Hatch-Waxman Act provides a 30-month stay, during which time the FDA cannot finally approve the generic's application. If the litigation is resolved in favor of the ANDA applicant during the 30-month stay period, the stay is lifted and the FDA's review of the application may be completed. The discovery, trial and appeals process in such a lawsuit is costly, time consuming, and may result in generic competition if the ANDA applicant prevails. Regardless of any litigation results, generic versions of Kuvan (sapropterin dihydrochloride) would be prohibited until the expiration of orphan drug exclusivity in June 2015, including pediatric exclusivity, at the earliest. We have also received three-year Hatch-Waxman exclusivity for a New Patient Population for Kuvan (sapropterin dihydrochloride) that expires in October 2017, including pediatric exclusivity. Thus, depending on the proposed labeling of a generic product, generic versions of Kuvan (sapropterin dihydrochloride) may be prohibited until October 2017, though it is possible that an ANDA applicant could propose to carve out information in the Kuvan labeling protected by the New Patient Population exclusivity and obtain approval earlier.

As previously disclosed, we have received a paragraph IV notice letter, dated October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying us that DRL has filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Additionally, we have received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying us that Par has filed an ANDA seeking

approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Together with Merck & Cie (Merck), we filed lawsuits against both DRL and Par in the United States District Court for the District of New Jersey alleging patent infringement for our patents relating to Kuvan triggering the automatic 30 month stay on the approval of each ANDA.

The filing of DRL's and Par's purported ANDAs in respect to Kuvan (sapropterin dihydrochloride) could have an adverse impact on our stock price, and litigation to enforce our patents is likely to cost a substantial amount and require significant management attention. If the patents covering Kuvan (sapropterin dihydrochloride) and its use are not upheld in litigation, or if DRL and/or Par is found to not infringe our asserted patents, the resulting generic competition following the expiration of regulatory exclusivity would have a material adverse effect on our revenue and results of operations.

If we do not achieve our projected development goals in the timeframes we announce and expect, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of scientific studies and clinical trials and the submission of regulatory filings. From time to time, we publicly announce the expected timing of some of these milestones. All of these milestones are based on a variety of assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in many cases for reasons beyond our control. If we do not meet these milestones as publicly announced, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

We depend upon our key personnel and our ability to attract and retain employees.

Our future growth and success will depend in large part on our continued ability to attract, retain, manage and motivate our employees. The loss of the services of any member of our senior management or the inability to hire or retain experienced management personnel could adversely affect our ability to execute our business plan and harm our operating results.

Because of the specialized scientific and managerial nature of our business, we rely heavily on our ability to attract and retain qualified scientific, technical and managerial personnel. In particular, the loss of one or more of our senior executive officers could be detrimental to us if we do not have an adequate succession plan or if we cannot recruit suitable replacements in a timely manner. While our senior executive officers are parties to employment agreements with us, these agreements do not guarantee that they will remain employed with us in the future. In addition, in many cases, these agreements do not restrict our senior executive officers' ability to compete with us after their employment is terminated. The competition for qualified personnel in the pharmaceutical field is intense, and there is a limited pool of qualified potential employees to recruit. Due to this intense competition, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to recruit suitable replacement personnel. If we are unsuccessful in our recruitment and retention efforts, our business may be harmed.

Our success depends on our ability to manage our growth.

Product candidates that we are currently developing or may acquire in the future may be intended for patient populations that are significantly larger than any of Mucopolysaccharidosis I (MPS I), Mucopolysaccharidosis VI (MPS VI), PKU or Lambert Eaton Myasthenic Syndrome (LEMS). In order to continue development and marketing of these products, if approved, we will need to significantly expand our operations. To manage expansion effectively, we need to continue to develop and improve our research and development capabilities, manufacturing and quality capacities, sales and marketing capabilities, financial and administrative systems and standard processes for global operations. Our staff, financial resources, systems, procedures or controls may be inadequate to support our operations and may increase our exposure to regulatory and corruption risks and our management may be unable to manage successfully future market opportunities or our relationships with customers and other third-parties.

Changes in methods of treatment of disease could reduce demand for our products and adversely affect revenues.

Even if our drug products are approved, if doctors elect a course of treatment which does not include our drug products, this decision would reduce demand for our drug products and adversely affect revenues. For example, if gene therapy becomes widely used as a treatment of genetic diseases, the use of enzyme replacement therapy, such as Naglazyme, Vimizim, and Aldurazyme in MPS diseases, could be greatly reduced. Changes in treatment method can

be caused by the introduction of other companies' products or the development of new technologies or surgical procedures which may not directly compete with ours, but which have the effect of changing how doctors decide to treat a disease.

If product liability lawsuits are successfully brought against us, we may incur substantial liabilities.

We are exposed to the potential product liability risks inherent in the testing, manufacturing and marketing of human pharmaceuticals. We currently maintain insurance against product liability lawsuits for the commercial sale of our products and for the clinical trials of our product candidates. Pharmaceutical companies must balance the cost of insurance with the level of coverage based on estimates of potential liability. Historically, the potential liability associated with product liability lawsuits for pharmaceutical products has been unpredictable. Although we believe that our current insurance is a reasonable estimate of our potential liability and represents a commercially reasonable balancing of the level of coverage as compared to the cost of the insurance, we may be subject to claims in connection with our clinical trials and commercial use of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse, or our

clinical trials for pegvaliase, reveglucosidase alfa, talazoparib, BMN 111, cerliponase alfa or BMN 270 for which our insurance coverage may not be adequate and we may be unable to avoid significant liability if any product liability lawsuit is brought against us. If we are the subject of a successful product liability claim that exceeds the limits of any insurance coverage we obtain, we may incur substantial charges that would adversely affect our earnings and require the commitment of capital resources that might otherwise be available for the development and commercialization of our product programs.

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

We rely significantly on our information technology and manufacturing infrastructure to effectively manage and maintain our inventory and internal reports, to manufacture and ship products to customers and to timely invoice them. Any failure, inadequacy or interruption of that infrastructure or security lapse of that technology, including cybersecurity incidents could harm our ability to operate our business effectively. Our ability to manage and maintain our inventory and internal reports, to manufacture and ship our products to customers and timely invoice them depends significantly on our enterprise resource planning, production management and other information systems. Cybersecurity attacks in particular are evolving and include, but are not limited to, malicious software, attempts to gain unauthorized access to data and other electronic security breaches that could lead to disruptions in systems, misappropriation of our confidential or otherwise protected information and corruption of data. Cybersecurity incidents resulting in the failure of our enterprise resource planning system, production management or other systems to operate effectively or to integrate with other systems, or a breach in security or other unauthorized access of these systems, may affect our ability to manage and maintain our inventory and internal reports, and result in delays in product fulfillment and reduced efficiency of our operations. A breach in security, unauthorized access resulting in misappropriation, theft, or sabotage with respect to our proprietary and confidential information, including research or clinical data, could require significant capital investments to remediate and could adversely affect our business, financial condition and results of operations.

*Our business is affected by macroeconomic conditions.

Various macroeconomic factors could adversely affect our business and the results of our operations and financial condition, including changes in inflation, interest rates and foreign currency exchange rates and overall economic conditions and uncertainties, including those resulting from the current and future conditions in the global financial markets. For instance, if inflation or other factors were to significantly increase our business costs, it may not be feasible to pass through price increases on to our customers due to the process by which health care providers are reimbursed for our products by the government. Interest rates, the liquidity of the credit markets and the volatility of the capital markets could also affect the value of our investments and our ability to liquidate our investments in order to fund our operations. We purchase or enter into a variety of financial instruments and transactions, including investments in commercial paper, the extension of credit to corporations, institutions and governments and hedging contracts. If any of the issuers or counter parties to these instruments were to default on their obligations, it could materially reduce the value of the transaction and adversely affect our cash flows.

For the three months ended March 31, 2015 approximately 4% of our net product revenues were from Italy, Spain, Portugal, Greece and Russia. Approximately 7% of our total accounts receivable as of March 31, 2015 related to such countries and we have included an allowance for doubtful accounts for certain accounts receivable from Greece. If the financial conditions of these countries continues to decline, a substantial portion of the receivables may be uncollectable, which would mean we would have to provide for additional allowances for doubtful accounts or cease selling products in these countries, either of which could adversely affect our results of operations. Additionally, if one or more of these countries were unable to purchase our products, our revenue would be adversely affected. We also sell our products in other countries that face economic crises and local currency devaluation. Although we have

historically collected receivables from customers in those countries, sustained weakness or further deterioration of the local economies and currencies may cause our customers in those countries to be unable to pay for our products with the same negative effect on our operations.

Interest rates and the ability to access credit markets could also adversely affect the ability of our customers/distributors to purchase, pay for and effectively distribute our products. Similarly, these macroeconomic factors could affect the ability of our contract manufacturers, sole-source or single-source suppliers to remain in business or otherwise manufacture or supply product. Failure by any of them to remain a going concern could affect our ability to manufacture products.

Recent and future regulatory actions and other events may adversely affect the trading price and liquidity of our senior subordinated convertible notes.

We expect that many investors in, and potential purchasers of, the Notes will employ, or seek to employ, a convertible arbitrage strategy with respect to the Notes. Investors would typically implement such a strategy by selling short the common stock underlying the Notes and dynamically adjusting their short position while continuing to hold the Notes. Investors may also implement this type of strategy by entering into swaps on our common stock in lieu of or in addition to short selling the common stock.

The SEC and other regulatory and self-regulatory authorities have implemented various rules and taken certain actions, and may in the future adopt additional rules and take other actions, that may impact those engaging in short selling activity involving equity securities (including our common stock). Such rules and actions include Rule 201 of SEC Regulation SHO, the adoption by the Financial Industry Regulatory Authority, Inc. of a “Limit Up-Limit Down” program, the imposition of market-wide circuit breakers that halt trading of securities for certain periods following specific market declines, and the implementation of certain regulatory reforms required by the Dodd-Frank Wall Street Reform and Consumer Protection Act of 2010. Any governmental or regulatory action that restricts the ability of investors in, or potential purchasers of, the Notes to effect short sales of our common stock or enter into swaps on our common stock could adversely affect the trading price and the liquidity of the Notes.

In addition, if investors and potential purchasers seeking to employ a convertible arbitrage strategy are unable to borrow or enter into swaps on our common stock, in each case on commercially reasonable terms, the trading price and liquidity of the Notes may be adversely affected.

Risks Related to our Acquisition of Prosensa Holding N.V.

If we do not successfully integrate Prosensa into our business operations, our business could be adversely affected.

We will need to successfully integrate the operations of Prosensa with our business operations. Integrating the operations of Prosensa with that of our own will be a complex and time-consuming process. Prior to the acquisition, Prosensa operated independently, with its own business, corporate culture, locations, employees and systems. There may be substantial difficulties, costs and delays involved in any integration of the business of Prosensa with that of our own. These may include:

·distracting management from day-to-day operations;

·potential incompatibility of corporate cultures;

·an inability to achieve synergies as planned;

·changes in the combined business due to potential divestitures or other requirements imposed by antitrust regulators;

·costs and delays in implementing common systems and procedures; and

·increased difficulties in managing our business due to the addition of international locations.

Many of these risks may be accentuated because the majority of Prosensa's operations, employees and customers are located outside of the U.S. Any one or all of these factors may increase operating costs or lower anticipated financial performance. Many of these factors are also outside of our control. Achieving anticipated synergies and the potential benefits underlying our reasons for the acquisition will depend on successful integration of the businesses. The failure to integrate the business operations of Prosensa successfully would have a material adverse effect on our business, financial condition and results of operations.

The actual impact of the acquisition on our capital structure and financial results may be worse than the assumptions we have used.

Even if the integration is successful, we have made certain assumptions relating to the impact on our capital structure and financial results in respect of the acquisition. These assumptions relate to numerous matters, including:

·our expected capital structure after the acquisition;

·the amount of goodwill and intangibles that will result from the acquisition;

- certain other purchase accounting adjustments that we expect will be recorded in our financial statements in connection with the acquisition;

- acquisition costs, including restructuring charges and transaction costs; and

- other financial and strategic risks of the acquisition.

Irrespective of our assumptions, we may incur higher than expected operating, transaction and integration costs, and we may encounter general economic and business conditions that adversely affect the combined company following the acquisition. If one or more of these assumptions are incorrect, it could have an adverse effect on our business and operating results, and the perceived benefits from the acquisition may not be realized.

We may have exposure to additional tax liabilities as a result of the acquisition.

As a multinational corporation, we are subject to income taxes as well as non-income based taxes, in both the U.S. and various foreign jurisdictions. Significant judgment is required in determining our worldwide provision for income taxes and other tax liabilities. Changes in tax laws or tax rulings may have a significantly adverse impact on our effective tax rate. Proposals by the current U.S. administration for fundamental U.S. international tax reform, including without limitation provisions that would limit the ability of U.S. multinationals to defer U.S. taxes on foreign income, if enacted, could have a significant adverse impact on our effective tax rate following the acquisition.

We are subject to a variety of additional risks as a result of the acquisition that may negatively impact our operations.

As a result of the acquisition, we are subject to new and additional risks associated with the business and operations of Prosensa and its global operations. The additional risks we may be exposed to include but are not limited to the following:

- tariffs and trade barriers;

- regulations related to customs and import/export matters (including sanctions);

- longer payment cycles;

- tax issues, such as tax law changes and variations in tax laws as compared to the jurisdictions in which we already operate;

- operating under regulations in new jurisdictions related to obtaining eligibility for government or private payer reimbursement for our products at the wholesale/retail level;

- cultural and language differences in the new jurisdictions in which we will operate;

·complying with additional employment regulations in the new jurisdictions in which we will operate; and

·risks related to crimes, strikes, riots, civil disturbances, terrorist attacks and wars in new geographical locations.

We cannot assure you that we will be able to adequately address these additional risks. If we are unable to do so, our operations might suffer.

Additionally, although prior to the acquisition we had international operations, as a result of the acquisition, we operate on an expanded global basis with additional offices or activities in Europe. We will face increased exposure to risks inherent in conducting business internationally, including compliance with international laws and regulations and laws and regulations of the U.S. and various other countries that apply to our international operations. Compliance with these laws and regulations may increase our cost of doing business in foreign jurisdictions. These laws and regulations include laws relating to the pharmaceutical industry, data privacy requirements, labor relations laws, tax laws, anti-competition regulations, import and trade restrictions, export requirements, U.S. laws such as the FCPA, other U.S. federal statutes and regulations, including those established by the Office of Foreign Assets Control, and local laws which prohibit payments to governmental officials. Given the high level of complexity of these laws, however, there is a risk that some provisions may be inadvertently breached by us, for example through fraudulent or negligent behavior of individual employees, our failure to comply with certain formal documentation requirements, or otherwise. Violations of these laws and regulations could result in fines, criminal sanctions against us, our officers or our employees, requirements to obtain export licenses, cessation of business activities in sanctioned countries, implementation of compliance programs, and prohibitions on the conduct of

our business. Any such violations could include prohibitions on our ability to offer our products in one or more countries and could materially damage our reputation, our brand, our international expansion efforts, our ability to attract and retain employees, our business and our operating results. Our success depends, in part, on our ability to anticipate these risks and manage these challenges. These factors or any combination of these factors may adversely affect our revenue or our overall financial performance.

We will incur significant transaction, integration and restructuring costs in connection with the acquisition.

We have incurred significant transaction costs related to the acquisition. In addition, the combined business will incur integration and restructuring costs following the completion of the acquisition as we integrate Prosensa's businesses with our businesses. Although we expect that the realization of benefits and efficiencies related to the integration of the businesses may offset over time these transaction and integration and restructuring costs, no assurances can be made that this net benefit will be achieved in the near term, or at all, which could adversely affect our financial condition and results of operations.

Prosensa depends heavily on the success of drisapersen. Drisapersen is still in clinical development. If we are unable to commercialize drisapersen or experience significant delays in doing so, our business, financial condition and results of operations will be materially adversely affected.

Our ability to generate product revenues from Prosensa will depend heavily on the successful development and eventual commercialization of drisapersen.

In September 2013, Prosensa announced that the Phase 3 clinical trial of drisapersen did not meet its primary endpoint. Although we believe that the collective data from Prosensa's various Phase 2 and Phase 3 clinical trials of drisapersen, including retrospective and subgroup analyses, provide strong support for concluding that drisapersen showed clinically meaningful improvements over placebo in these trials, we cannot be sure that Prosensa's data will be sufficient to satisfy the EMA or the FDA. We may need to conduct additional clinical trials at significant delay and cost or abandon development of drisapersen altogether.

Even if we receive regulatory approval for and are able to commercialize drisapersen, our success will be subject to the following risks:

- we may not achieve market acceptance of drisapersen by physicians, patients and third-party payers;
- drisapersen may not have an acceptable safety profile following approval;
- we may not be able to manufacture drisapersen in compliance with requirements of the EMA, the FDA and similar regulatory agencies in commercial quantities sufficient to meet market demand;
- we may not achieve sufficient pricing for drisapersen to compensate for future development and commercialization costs and to recoup our cost to acquire Prosensa;
- we may not compete successfully with any alternative therapies for Duchenne muscular dystrophy (DMD); and

· we may not successfully enforce and defend our intellectual property rights and claims.

The occurrence of any of these events could materially adversely affect our business, financial condition and results of operations.

Our conclusions regarding the efficacy of drisapersen are based on retrospective analyses of the results of Prosensa's clinical trials, and these analyses may be considered less reliable indicators of efficacy than pre-specified analyses.

After determining that it did not achieve the primary efficacy endpoint in the completed Phase 3 clinical trial of drisapersen, Prosensa performed retrospective and subgroup analyses of the Phase 3 clinical trial and prior Phase 2 clinical trials of drisapersen that we believe provide strong support for concluding that drisapersen showed clinically meaningful improvements over placebo in these trials. Although Prosensa believed that these additional analyses were warranted, a retrospective analysis performed after unblinding trial results can result in the introduction of bias if the analysis is inappropriately tailored or influenced by knowledge of the data and actual results. Because of these limitations, regulatory authorities typically give greatest weight to results from pre-specified analyses and less weight to results from post-hoc, retrospective analyses. Thus, this increases the likelihood that we will have to conduct an additional clinical trial or trials of drisapersen before we can apply for marketing approval.

Because Prosensa was developing product candidates for the treatment of diseases in which there is little clinical experience and, in some cases, using new endpoints or methodologies, there is more risk that the outcome of clinical trials for Prosensa's product candidates will not be favorable.

There is currently no approved disease-modifying therapy for DMD. In addition, there has been limited historical clinical trial experience generally for the development of drugs to treat the underlying cause of DMD. As a result, the design and conduct of clinical trials for this disease, particularly for drugs to address the underlying cause of this disease, are subject to increased risks. In particular, regulatory authorities in the U.S. and the EU have not issued definitive guidance as to how to measure and achieve efficacy.

In the last several years, the six-minute walk test (6MWT) has been used in several trials of product candidates for patients with DMD, and is accepted by U.S. and European regulators to be an appropriate primary outcome measure for DMD trials. Because of the limited clinical experience in this indication however, regulators have not yet established what difference in the six-minute walk distance (6MWD) is required to be demonstrated in a clinical trial of a DMD therapy in order to signify a clinically meaningful result and/or obtain regulatory approvals. As a result, it is not clear what is required in terms of 6MWD or other end points to obtain regulatory approval for drisapersen and our other product candidates acquired from Prosensa. If we are required to conduct additional clinical trials of drisapersen, the design of such trials could be subject to such uncertainties.

We could also face similar challenges in designing clinical trials and obtaining regulatory approval for future product candidates, including any that we may develop for myotonic dystrophy or Huntington's disease because there is also limited historical clinical trial experience for the development of drugs to treat these diseases.

Risks Related to Ownership of Our Securities

Our stock price may be volatile, and an investment in our stock could suffer a decline in value.

Our valuation and stock price since the beginning of trading after our initial public offering have had no meaningful relationship to current or historical earnings, asset values, book value or many other criteria based on conventional measures of stock value. The market price of our common stock will fluctuate due to factors including:

- product sales and profitability of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- manufacture, supply or distribution of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- progress of our integration of Prosensa;
- progress of our product candidates through the regulatory process and our ability to successfully commercialize any such products that receive regulatory approval;
- results of clinical trials, announcements of technological innovations or new products by us or our competitors;

- results relating to our lawsuit against DRL to protect our patents relating to Kuvan;

- government regulatory action affecting our product candidates or our competitors' drug products in both the U.S. and non-U.S. countries;

- developments or disputes concerning patent or proprietary rights;

- general market conditions and fluctuations for the emerging growth and pharmaceutical market sectors;

- economic conditions in the U.S. or abroad;

- broad market fluctuations in the U.S., the EU or in other parts of the world;

- actual or anticipated fluctuations in our operating results; and

- changes in company assessments or financial estimates by securities analysts.

In the past, following periods of large price declines in the public market price of a company's securities, securities class action litigation has often been initiated against that company. Litigation of this type could result in substantial costs and diversion of management's attention and resources, which would hurt our business. Any adverse determination in litigation could also subject us to significant liabilities. In addition, our stock price can be materially adversely affected by factors beyond our control, such as disruptions in global financial markets or negative trends in the biotechnology sector of the economy, even if our business is operating well.

Anti-takeover provisions in our charter documents and under Delaware law may make an acquisition of us, which may be beneficial to our stockholders, more difficult.

We are incorporated in Delaware. Certain anti-takeover provisions of Delaware law and our charter documents as currently in effect may make a change in control of our company more difficult, even if a change in control would be beneficial to the stockholders. Our anti-takeover provisions include provisions in our certificate of incorporation providing that stockholders' meetings may only be called by our Board of Directors and provisions in our bylaws providing that the stockholders may not take action by written consent and requiring that stockholders that desire to nominate any person for election to our Board of Directors or to make any proposal with respect to business to be conducted at a meeting of our stockholders be submitted in appropriate form to our Secretary within a specified period of time in advance of any such meeting. Additionally, our Board of Directors has the authority to issue shares of preferred stock and to determine the terms of those shares of stock without any further action by our stockholders. The rights of holders of our common stock are subject to the rights of the holders of any preferred stock that may be issued. The issuance of preferred stock could make it more difficult for a third-party to acquire a majority of our outstanding voting stock. Delaware law also prohibits corporations from engaging in a business combination with any holders of 15% or more of their capital stock until the holder has held the stock for three years unless, among other possibilities, our Board of Directors approves the transaction. Our Board of Directors may use these provisions to prevent changes in the management and control of our company. Also, under applicable Delaware law, our Board of Directors may adopt additional anti-takeover measures in the future.

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

During the three months ended March 31, 2015, the Company entered into separate agreements with three existing holders of its 2017 Notes pursuant to which such holders converted $8.1 million in aggregate principal amount of the 2017 Notes into 399,469 shares of the Company's common stock. In addition to issuing the requisite number of shares of the Company's common stock, the Company also made varying cash payments to the holder totaling approximately $0.2 million in the aggregate. The issuance of the common stock of the Company upon conversion of the 2017 Notes was made in reliance on the exemption from the registration requirements of the Securities Act of 1933, as amended, pursuant to Section 3(a)(9) thereof, as the conversion of the 2017 Notes into common stock of the Company was made by the Company with its existing security holders exclusively in a series of privately negotiated transactions where no commission or other remuneration was paid.

Item 3. Defaults Upon Senior Securities.
None.

Item 4. Mine Safety Disclosures
None.

Item 5. Other Information.
None.

Item 6. Exhibits.

31.1*	Certification of Chief Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
31.2*	Certification of Chief Financial Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
32.1*	Certification of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. This Certification accompanies this report and shall not, except to the extent required by the Sarbanes-Oxley Act of 2002, be deemed filed for purposes of §18 of the Securities Exchange Act of 1934, as amended.
101.INS*	XBRL Instance Document
101.SCH*	XBRL Taxonomy Extension Schema Document
101.CAL*	XBRL Taxonomy Extension Calculation Document
101.DEF*	XBRL Taxonomy Extension Definition Linkbase
101.LAB*	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE*	XBRL Taxonomy Extension Presentation Link Document

*Filed herewith.

Attached as Exhibit 101 to this report are documents formatted in XBRL (Extensible Business Reporting Language): (i) Condensed Consolidated Balance Sheets as of March 31, 2015 and December 31, 2014, (ii) Condensed Consolidated Statements of Comprehensive Loss for the three months ended March 31, 2015 and 2014, (iii) Condensed Consolidated Statements of Cash Flows for the three months ended March 31, 2015 and 2014, and (iv) Notes to Condensed Consolidated Financial Statements.

SIGNATURE

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

BIOMARIN PHARMACEUTICAL INC.

Dated: May 5, 2015

By /S/ DANIEL SPIEGELMAN
Daniel Spiegelman,

Executive Vice President and Chief Financial Officer
(On behalf of the registrant and as principal financial officer)

EXHIBIT INDEX

31.1*	Certification of Chief Executive Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
31.2*	Certification of Chief Financial Officer pursuant to Rules 13a-14(a)/15d-14(a) of the Securities Exchange Act of 1934, as amended.
32.1*	Certification of Chief Executive Officer and Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. This Certification accompanies this report and shall not, except to the extent required by the Sarbanes-Oxley Act of 2002, be deemed filed for purposes of §18 of the Securities Exchange Act of 1934, as amended.
101.INS*	XBRL Instance Document
101.SCH*	XBRL Taxonomy Extension Schema Document
101.CAL*	XBRL Taxonomy Extension Calculation Document
101.DEF*	XBRL Taxonomy Extension Definition Linkbase
101.LAB*	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE*	XBRL Taxonomy Extension Presentation Link Document

*Filed herewith.

Attached as Exhibit 101 to this report are documents formatted in XBRL (Extensible Business Reporting Language): (i) Condensed Consolidated Balance Sheets as of March 31, 2015 and December 31, 2014, (ii) Condensed Consolidated Statements of Comprehensive Loss for the three months ended March 31, 2015 and 2014,

(iii) Condensed Consolidated Statements of Cash Flows for the three months ended March 31, 2015 and 2014, and
(iv) Notes to Condensed Consolidated Financial Statements.