

BIOMARIN PHARMACEUTICAL INC

Form 10-Q

May 02, 2016

o

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the quarterly period ended March 31, 2016

Or

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the transition period from \_\_\_\_\_ to \_\_\_\_\_ .

Commission File Number: 000-26727

BioMarin Pharmaceutical Inc.

(Exact name of registrant as specified in its charter)

Delaware 68-0397820  
(State or other jurisdiction of (I.R.S. Employer

incorporation or organization) Identification No.)

770 Lindero 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  No

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

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

Large accelerated filer

Accelerated filer

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

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

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  No

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: 162,322,439 shares of common stock, par value \$0.001, outstanding as of April 22, 2016.

BIOMARIN PHARMACEUTICAL INC.

TABLE OF CONTENTS

	Page
PART I. FINANCIAL INFORMATION	
Item 1. Financial Statements	
<u>Condensed Consolidated Balance Sheets as of March 31, 2016 (Unaudited) and December 31, 2015</u>	3
<u>Condensed Consolidated Statements of Comprehensive Loss (Unaudited) for the three months ended March 31, 2016 and 2015</u>	4
<u>Condensed Consolidated Statement of Stockholders' Equity (Unaudited) for the three months ended March 31, 2016</u>	5
<u>Condensed Consolidated Statements of Cash Flows (Unaudited) for the three months ended March 31, 2016 and 2015</u>	6
<u>Notes to Condensed Consolidated Financial Statements (Unaudited)</u>	7
Item 2. <u>Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	27
Item 3. <u>Quantitative and Qualitative Disclosures about Market Risk</u>	40
Item 4. <u>Controls and Procedures</u>	40
PART	
II. <u>OTHER INFORMATION</u>	40
Item 1. <u>Legal Proceedings</u>	40
Item 1A. <u>Risk Factors</u>	40
Item 2. <u>Unregistered Sales of Equity Securities and Use of Proceeds</u>	63
Item 3. <u>Defaults Upon Senior Securities</u>	63
Item 4. <u>Mine Safety Disclosures</u>	63
Item 5. <u>Other Information</u>	63
Item 6. <u>Exhibits</u>	63
<u>SIGNATURES</u>	64

Kysdrisa™ is our trademark. BioMarin®, Vimizim®, Naglazyme®, Kuvan® and Firdapse® are our registered trademarks. Aldurazyme® is a registered trademark of BioMarin/Genzyme LLC. All other brand names and service marks, trademarks and other trade names appearing in this report are the property of their respective owners.

## BIOMARIN PHARMACEUTICAL INC.

## CONDENSED CONSOLIDATED BALANCE SHEETS

March 31, 2016 and December 31, 2015

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

	March 31, 2016	December 31, 2015 <sup>(1)</sup>
<b>ASSETS</b>	(unaudited)	
Current assets:		
Cash and cash equivalents	\$ 270,453	\$ 397,040
Short-term investments	186,400	195,579
Accounts receivable, net (allowance for doubtful accounts: \$167 and \$93, at March 31, 2016 and December 31, 2015, respectively)	180,751	164,959
Inventory	296,979	271,683
Other current assets	58,207	60,378
Total current assets	992,790	1,089,639
Noncurrent assets:		
Long-term investments	314,404	425,652
Property, plant and equipment, net	716,916	704,207
Intangible assets, net	1,177,232	683,996
Goodwill	197,039	197,039
Deferred tax assets	243,212	220,191
Other assets	25,400	408,644
Total assets	\$ 3,666,993	\$ 3,729,368
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 290,562	\$ 392,511
Short-term contingent acquisition consideration payable	97,449	52,946
Total current liabilities	388,011	445,457
Noncurrent liabilities:		
Long-term convertible debt, net	668,009	662,286
Long-term contingent acquisition consideration payable	135,275	32,663
Deferred tax liabilities	143,527	143,527
Other long-term liabilities	41,935	44,588
Total liabilities	1,376,757	1,328,521
Stockholders' equity:		
Common stock, \$0.001 par value: 250,000,000 shares authorized at  March 31, 2016 and December 31, 2015: 162,243,016 and 161,526,044 shares  issued and outstanding at March 31, 2016 and December 31, 2015, respectively	163	162
Additional paid-in capital	3,410,297	3,414,837
Company common stock held by Nonqualified Deferred Compensation Plan	(13,560 )	(13,616 )
Accumulated other comprehensive income	47	21,033
Accumulated deficit	(1,106,711)	(1,021,569 )

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 10-Q

Total stockholders' equity	2,290,236	2,400,847
Total liabilities and stockholders' equity	\$3,666,993	\$3,729,368

(1) December 31, 2015 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, 2015, filed with the Securities and Exchange Commission (the SEC) on February 29, 2016.

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, 2016 and 2015

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

(Unaudited)

	Three Months Ended March 31,	
	2016	2015
<b>REVENUES:</b>		
Net product revenues	\$235,357	\$201,312
Collaborative agreement revenues	233	376
Royalty, license and other revenues	1,146	1,232
Total revenues	236,736	202,920
<b>OPERATING EXPENSES:</b>		
Cost of sales	43,118	30,998
Research and development	158,793	142,074
Selling, general and administrative	105,300	92,806
Intangible asset amortization and contingent consideration	10,442	2,902
Total operating expenses	317,653	268,780
LOSS FROM OPERATIONS	(80,917 )	(65,860 )
Equity in the loss of BioMarin/Genzyme LLC	(135 )	(150 )
Interest income	1,571	683
Interest expense	(9,843 )	(9,462 )
Debt conversion expense	—	(163 )
Other income	198	249
LOSS BEFORE INCOME TAXES	(89,126 )	(74,703 )
Benefit from income taxes	(3,984 )	(7,202 )
NET LOSS	\$(85,142 )	\$(67,501 )
NET LOSS PER SHARE, BASIC AND DILUTED	\$(0.53 )	\$(0.43 )
Weighted average common shares outstanding, basic and diluted	161,548	157,612
<b>COMPREHENSIVE LOSS</b>	<b>\$(106,128)</b>	<b>\$(51,148 )</b>

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

## BIOMARIN PHARMACEUTICAL INC.

## CONDENSED CONSOLIDATED STATEMENT OF STOCKHOLDERS' EQUITY

Three Months Ended March 31, 2016

(In thousands of U.S. dollars)

(Unaudited)

	Common stock		Additional Paid-in Capital	Company Common Stock Held by Nonqualified Deferred Compensation Plan	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Capital	Plan	(Loss)	Deficit	Equity
Balance at December 31, 2015	161,526	\$ 162	\$3,414,837	\$ (13,616 )	\$ 21,033	\$ (1,021,569 )	\$ 2,400,847
Net loss						(85,142 )	(85,142 )
Other comprehensive loss					(20,986 )		(20,986 )
Exercise of common stock options	114		3,521				3,521
Excess tax benefit from stock option exercises			99				99
Restricted stock vested during the period, net	524	1	(40,789 )				(40,788 )
Conversion of convertible notes, net	79		1,614				1,614
Common stock held by Nonqualified							
Deferred Compensation Plan (the NQDC)				56			56
Stock-based compensation			31,015				31,015
Balance at March 31, 2016	162,243	\$ 163	\$3,410,297	\$ (13,560 )	\$ 47	\$ (1,106,711 )	\$ 2,290,236

## BIOMARIN PHARMACEUTICAL INC.

## CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

Three Months Ended March 31, 2016 and 2015

(In thousands of U.S. dollars)

(Unaudited)

	2016	2015
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>		
Net loss	\$(85,142 )	\$(67,501 )
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	20,244	10,817
Non-cash interest expense	7,337	7,000
Accretion of discount on investments	309	417
Stock-based compensation	30,177	22,692
Deferred income taxes	(19,277 )	(7,800 )
Excess tax benefit from stock option exercises	(99 )	(527 )
Unrealized foreign exchange gain on forward contracts	(6,526 )	(5,686 )
Non-cash changes in the fair value of contingent acquisition consideration payable	2,936	282
Other	526	(130 )
Changes in operating assets and liabilities:		
Accounts receivable, net	(16,044 )	(26,789 )
Inventory	(11,803 )	(19,344 )
Other current assets	2,399	(3,522 )
Other assets	(1,232 )	330
Accounts payable and accrued liabilities	(90,744 )	(57,236 )
Other long-term liabilities	(4,614 )	9,186
Net cash used in operating activities	(171,553)	(137,811)
<b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>		
Purchases of property, plant and equipment	(45,204 )	(43,832 )
Maturities and sales of investments	181,267	124,137
Purchase of available-for-sale investments	(58,914 )	(288,431)
Purchase of promissory note	(150 )	(3,326 )
Business acquisitions, net of cash acquired	—	(538,392)
Other	—	(1,027 )
Net cash provided by (used in) investing activities	76,999	(750,871)
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>		
Proceeds from exercises of stock options and Employee Stock Purchase Plan (the ESPP)	3,521	28,026
Taxes paid related to net share settlement of equity awards	(40,788 )	(735 )
Proceeds from public offering of common stock, net	—	888,257
Excess tax benefit from stock option exercises	99	527
Other	3	(1,284 )
Net cash provided by (used in) financing activities	(37,165 )	914,791
Effect of exchange rate changes on cash	5,132	(1,025 )
<b>NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS</b>	<b>(126,587)</b>	<b>25,084</b>
Cash and cash equivalents:		



Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 10-Q

Beginning of period	\$397,040	\$875,486
End of period	\$270,453	\$900,570
SUPPLEMENTAL CASH FLOW DISCLOSURES:		
Cash paid for income taxes	73,215	1,358
Stock-based compensation capitalized into inventory	2,469	2,480
Depreciation capitalized into inventory	2,617	3,580
SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON CASH INVESTING AND FINANCING		
ACTIVITIES:		
Decrease in accounts payable and accrued liabilities related to fixed assets	(14,788 )	(20,985 )
Conversion of convertible debt	—	8,133
Accrual for inventory purchases related to the acquisition of the Merck PKU Business	2,436	—
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 consists of five approved products and multiple clinical and pre-clinical product candidates. The Company's approved products are Vimizim (elosulfase alfa), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (aronidase) and Firdapse (amifampridine phosphate).

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 its approved products; 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 the United States (U.S.) generally accepted accounting principles (U.S. GAAP) for complete financial statements, although the Company believes that the disclosures herein are adequate to ensure that the information presented is not misleading. 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, 2015 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, 2016 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2016.

Management performed an evaluation of the Company's activities through the date of filing of this Quarterly Report on Form 10-Q, and has concluded that there were no subsequent events or transactions that occurred subsequent to the balance sheet date 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, 2016, 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, 2015.

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

Except as described below, there have been no new accounting pronouncements or changes to accounting pronouncements during the three months ended March 31, 2016, 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, 2015, that are of significance or potential significance to the Company.

In March 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2016-09, Improvements to Employee Share-Based Payment Accounting (ASU 2016-09), which is intended to simplify several aspects of the accounting for share-based payment transactions, including the income tax consequences, classification of awards as either equity or liabilities, and classification on the statement of cash flows. ASU 2016-09 is effective for fiscal years beginning after December 15, 2016, including interim periods within those fiscal years. Early application is permitted. ASU 2016-09 will be effective for the Company's fiscal year beginning January 1, 2017 unless it elects early adoption. The Company is currently evaluating the potential impact the adoption of ASU 2016-09 will have on its consolidated financial statements and has not elected to early adopt the amendments.

In February 2016, the FASB issued ASU No. 2016-02, Leases (ASU 2016-02). The amended guidance requires balance sheet recognition of lease assets and liabilities by lessees for leases classified as operating leases, with an option to not recognize lease assets and lease liabilities for leases with a term of 12 months or less. The amendments also require new disclosures providing additional qualitative and quantitative information about the amounts recorded in the financial statements. Lessor accounting is largely unchanged. ASU 2016-02 is effective for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years. Early application is permitted. ASU 2016-02 will be effective for the Company's fiscal year beginning January 1, 2019 unless it elects early adoption. The amendments require a modified retrospective approach with optional practical expedients. The Company is currently evaluating the potential impact the adoption of ASU 2016-02 will have on its consolidated financial statements and has not elected to early adopt ASU 2016-02.

In May 2014, the FASB issued ASU No. 2014-09 (ASU 2014-09) regarding Accounting Standards Codification (ASC) Topic 606, Revenue from Contracts with Customers. ASU 2014-09 provides principles for recognizing revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the Company expects to be entitled in exchange for those goods or services. In August 2015, the FASB issued ASU 2015-14 to defer the effective date by one year with early adoption permitted as of the original effective date. ASU 2014-09 will be effective for the Company's fiscal year beginning January 1, 2018 unless it elects the earlier date of January 1, 2017. In March 2016, the FASB issued ASU 2016-08 to help provide interpretive clarifications on the new guidance for ASC Topic 606. In April 2016, the FASB issued ASU 2016-10 to clarify the guidance for identifying performance obligations and accounting for licenses of intellectual property. The Company is currently evaluating the accounting, transition, and disclosure requirements of the standard.

(5) ACQUISITIONS

The Merck PKU Business

On October 1, 2015 the Company entered into a Termination and Transition Agreement with Ares Trading S.A. (Merck Serono), as amended and restated on December 23, 2015 (the A&R Kuvan Agreement), to terminate the Development, License and Commercialization Agreement, dated May 13, 2005, as amended (the License Agreement), between the Company and Merck Serono, including the license to Kuvan granted in the License Agreement from the Company to Merck Serono. Also on October 1, 2015, the Company and Merck Serono entered into a Termination Agreement (the Pegvaliase Agreement) to terminate the license to pegvaliase granted in the License Agreement from the Company to Merck Serono. On January 1, 2016, pursuant to the A&R Kuvan Agreement and the Pegvaliase Agreement, the Company completed the acquisition from Merck Serono and its affiliates of certain rights and other assets with respect to Kuvan and pegvaliase (the Merck PKU Business). As a result, the Company acquired all global rights to Kuvan and pegvaliase from Merck Serono, with the exception of Kuvan in Japan. Previously, the Company had exclusive rights to Kuvan in the U.S. and Canada and pegvaliase in the U.S. and Japan. In connection with the acquisition of Merck PKU Business, the Company recognized transaction costs of \$0.4 million, of which \$0.3 million and \$0.1 million, respectively, were recognized in the year ended December 31, 2015 and the three months ended March 31, 2016.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

Pursuant to the A&R Kuvan Agreement, the Company paid Merck Serono \$374.2 million, in cash, and is obligated to pay Merck Serono up to a maximum of €60.0 million, in cash, if future sales milestones are met. Pursuant to the Pegvaliase Agreement, the Company is obligated to pay Merck Serono up to a maximum of €125.0 million, in cash, if future development milestones are met. Merck Serono transferred certain inventory, regulatory materials and approvals, and intellectual property rights to the Company and will perform certain transition services for the Company.

The Company and Merck Serono have no further rights or obligations under the License Agreement with respect to pegvaliase. The License Agreement will continue in effect in order to complete the transfer of certain assets related to Kuvan, the majority of which occurred in January 2016. Accordingly, as of March 31, 2016, the Company continues to rely on Merck Serono to provide critical transition services for the sales and distribution of Kuvan until marketing authorizations can be transferred in approximately 12 remaining countries, but in no event later than December 31, 2016.

Prior to the consummation of the transactions described above, the Company sold Kuvan to Merck Serono at a price near its manufacturing costs, and Merck Serono resold the product to end users outside the U.S., Canada and Japan. The royalty earned by the Company from Kuvan product sold by Merck Serono was included as a component of Net Product Revenues in the period earned.

Kuvan is a commercialized product for the treatment of patients with phenylketonuria (PKU). Pegvaliase is currently in registration-enabling pivotal studies as a potential therapeutic option for adult patients with PKU. Kuvan has Orphan Drug exclusivity in Europe until 2020 and pegvaliase has Orphan Drug designation in the U.S. and European Union (EU).

The acquisition date fair value of the contingent acquisition consideration payments, Kuvan global marketing rights, with the exception of Japan, and pegvaliase in-process research and development (IPR&D) acquired was estimated by applying a probability-based income approach utilizing an appropriate discount rate. This estimation was based on significant inputs that are not observable in the market, referred to as level 3 inputs. Key assumptions include a discount rate and various probability factors. The range of outcomes and assumptions used to develop these estimates has been updated to estimate the fair value of the contingent acquisition consideration payable at March 31, 2016. See Note 14 to these Condensed Consolidated Financial Statements for additional discussion regarding fair value measurements of the contingent acquisition consideration payable included on the Company's Condensed Consolidated Balance Sheet.

The following table presents the preliminary allocation of the purchase consideration for the Merck PKU Business acquisition, including the contingent acquisition consideration payable, based on the acquisition date fair value:

Cash payments	\$374,192
Estimated fair value of contingent acquisition consideration payable	138,974
Total consideration	\$513,166

Kuvan intangible assets	\$173,486
Pegvaliase IPR&D	327,350
Inventory	12,330
Total identifiable assets acquired	\$513,166

The amount allocated to the Kuvan intangible assets is considered to be finite-lived and will be amortized on a straight-line basis over its estimated useful life through 2024.

## 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 amount allocated to acquired pegvaliase IPR&D is considered to be indefinite-lived until the completion or abandonment of the associated research and development 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 the reduction in the fair value of the IPR&D assets below their respective carrying amounts. 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. See Note 8 to these Condensed Consolidated Financial Statements for further discussion of the indefinite-lived intangible assets.

## Pro Forma Financial Information

The following unaudited pro forma financial information presents the combined results of operations of the Company and the Merck PKU Business as if the acquisition occurred on January 1, 2015. This unaudited pro forma financial information is presented for informational purposes only and is not necessarily indicative of the results of future operations that would have been achieved had the acquisitions taken place at the beginning of 2015.

	Three Months Ended March 31, 2015
Total revenues	\$218,798
Net loss	\$(61,281 )
Net loss per share, basic and dilutive	\$(0.39 )
Weighted average common shares outstanding, basic and diluted	157,612

## (6) 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 ESPP, unvested restricted stock units (RSUs), common stock held by the NQDC and contingent issuances of common stock related to convertible debt. The table below presents the shares of common stock that were excluded from the computation as they were anti-dilutive using the



treasury stock method (in thousands of shares):

	Three Months Ended March 31,	
	2016	2015
Options to purchase common stock	10,884	11,109
Common stock issuable under the 2017 Notes	1,464	1,567
Common stock issuable under the 2018 and 2020 Notes	7,966	7,966
Unvested restricted stock units	1,529	1,557
Potentially issuable common stock for ESPP purchases	151	223
Common stock held by the NQDC	238	213
Total number of potentially issuable shares	22,232	22,635

The effect of the Company's 0.7% 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) were excluded from the diluted net loss per common share because 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, 2016 and 2015, the Notes had no effect on diluted net loss per share as the Company's stock price did not exceed the conversion price of \$94.15 per share for the Notes.

## 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) INVESTMENTS

All investments were classified as available-for-sale at March 31, 2016 and December 31, 2015. 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, 2016 and December 31, 2015 are summarized in the tables below:

	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value at March 31, 2016
Certificates of deposit	\$ 11,517	\$ —	\$ —	\$ 11,517
Corporate debt securities	321,358	1,161	(164 )	322,355
Commercial paper	6,247	—	—	6,247
U.S. government agency securities	160,376	210	(25 )	160,561
Greek government-issued bonds	47	77	—	124
Total	\$ 499,545	\$ 1,448	\$ (189 )	\$ 500,804

	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Aggregate Fair Value at December 31, 2015
Certificates of deposit	\$ 63,919	\$ 1	\$ —	\$ 63,920
Corporate debt securities	358,625	20	(732 )	357,913
Commercial paper	12,733	—	—	12,733
U.S. government agency securities	186,882	—	(344 )	186,538
Greek government-issued bonds	48	79	—	127
Total	\$ 622,207	\$ 100	\$ (1,076 )	\$ 621,231

The Company has two investments in marketable equity securities measured using quoted prices in their respective active markets that are collectively considered strategic investments. As of March 31, 2016, the fair value of the Company's marketable equity securities was \$8.1 million, which included an unrealized gain of \$2.8 million. As of December 31, 2015, the fair value of the Company's marketable equity securities was \$18.1 million, which included an unrealized gain of \$12.7 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, 2016	December 31, 2015
Maturing in one year or less	\$186,400	\$ 195,579
Maturing after one year through five years	314,404	425,652
Total	\$500,804	\$ 621,231

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, 2016, 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 13 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)

## (8) INTANGIBLE ASSETS

Intangible assets consisted of the following:

	March 31, 2016	December 31, 2015
<b>Intangible assets:</b>		
Finite-lived intangible assets	\$ 302,595	\$ 129,572
Indefinite-lived intangible assets	935,361	607,548
Gross intangible assets:	1,237,956	737,120
Less: Accumulated amortization	(60,724 )	(53,124 )
Net carrying value	\$ 1,177,232	\$ 683,996

## Indefinite-Lived Intangible Assets

Intangible assets related to 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 8 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2015 for additional information related to the Company's intangible assets.

## (9) PROPERTY, PLANT AND EQUIPMENT

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

	March 31, 2016	December 31, 2015
Building and improvements	\$ 500,299	\$ 442,100

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 10-Q

Manufacturing and laboratory equipment	201,168	145,313
Computer hardware and software	118,563	113,442
Leasehold improvements	44,074	44,247
Furniture and equipment	24,714	22,817
Land improvements	4,881	4,881
Land	45,727	45,727
Construction-in-progress	71,607	164,283
	1,011,033	982,810
Less: Accumulated depreciation	(294,117 )	(278,603 )
Total property, plant and equipment, net	\$716,916	\$ 704,207

Construction in-process primarily includes costs related to the Company's significant in-process projects at its campus in Marin County, California, and its manufacturing facility in Shanbally, Cork, Ireland.

Depreciation expense for the three months ended March 31, 2016 and 2015 was \$15.7 million and \$11.5 million, respectively, of which \$2.6 million and \$3.6 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, 2016 and 2015 was insignificant.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## (10) SUPPLEMENTAL BALANCE SHEET INFORMATION

Inventory consisted of the following:

	March 31, 2016	December 31, 2015
Raw materials	\$44,973	\$ 46,115
Work-in-process	163,333	150,289
Finished goods	88,673	75,279
Total inventory	\$296,979	\$ 271,683

In the first quarter of 2016, process qualification production activities commenced in the Company's Shanbally facility related to Vimizim production. As of March 31, 2016 the value of the qualification campaign was \$15.5 million as of March 31, 2016 and which was capitalized as inventory because the product is expected to be sold commercially. While the Company believes it is unlikely that the manufacturing process will not be approved for Vimizim production, should that occur, the value of the inventory would be expensed at that time.

Other Assets consisted of the following:

	March 31, 2016	December 31, 2015
Deposit for business acquisition	\$ —	\$ 371,756
Deposits	8,720	8,606
Strategic investments	8,142	18,056
Long-term forward foreign currency exchange contract assets	734	3,533
Other	7,804	6,693
Total other assets	\$ 25,400	\$ 408,644

Accounts Payable and Accrued Liabilities consisted of the following:

	March 31, 2016	December 31, 2015
Accounts payable and accrued operating expenses	\$ 152,860	\$ 179,294
Accrued compensation expense	45,031	78,424
Accrued rebates payable	34,158	32,553

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 10-Q

Accrued vacation expense	19,696	16,921
Accrued royalties payable	10,787	10,412
Value added taxes payable	10,437	6,377
Accrued income taxes	1,593	59,572
Other	16,000	8,958
Total accounts payable and accrued liabilities	\$ 290,562	\$ 392,511

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## (11) CONVERTIBLE DEBT

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

	March 31, 2016	December 31, 2015
Convertible Notes due 2020	\$374,993	\$374,993
Unamortized discount	(62,483 )	(65,478 )
Unamortized deferred offering costs	(5,889 )	(6,210 )
Convertible Notes due 2020, net	306,621	303,305
Convertible Notes due 2018	374,980	374,980
Unamortized discount	(38,387 )	(41,904 )
Unamortized deferred offering costs	(4,933 )	(5,415 )
Convertible Notes due 2018, net	331,660	327,661
Convertible Notes due 2017	29,816	31,430
Unamortized deferred offering costs	(88 )	(110 )
Convertible Notes due 2017, net	29,728	31,320
Total convertible debt, net	\$668,009	\$662,286
Fair value of fixed rate convertible debt		
Convertible Notes due in 2020 <sup>(1)</sup>	\$441,884	\$502,701
Convertible Notes due in 2018 <sup>(1)</sup>	435,033	482,584
Convertible Notes due in 2017 <sup>(1)</sup>	119,780	162,016
Total	\$996,697	\$1,147,301

(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 consisted of the following:

	Three Months Ended March 31,	
	2016	2015
Coupon interest	\$2,506	\$2,462
Amortization of issuance costs	825	826
Accretion of debt discount	6,512	6,174
Total interest expense on convertible debt	\$9,843	\$9,462

During the three months ended March 31, 2015, the Company entered into three separate agreements with existing holders of its senior subordinated 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 \$0.2 million in the aggregate, of which \$0.2 million was recognized in total as Debt Conversion Expense on the Company's Condensed Consolidated Statement of Comprehensive Loss for the three months ended March 31, 2015.

See Note 14 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2015 for additional information related to the Company's convertible debt.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

(12) 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. Information regarding the specific instruments used by the Company to hedge its exposure to foreign currency exchange rate fluctuations is provided below. See Note 13 to these Condensed Consolidated Financial Statements for additional discussion regarding the fair value of forward foreign currency exchange contracts.

At March 31, 2016, the Company had 230 forward foreign currency exchange contracts outstanding to sell a total of 283.4 million Euros, 131 forward foreign currency exchange contracts outstanding to purchase 104.0 million Euros, 18 forward foreign currency exchange contracts outstanding to sell 9.4 million Canadian Dollars, 9 forward foreign currency exchange contracts outstanding to sell 29.4 billion Colombian Pesos, and one forward foreign currency exchange contract to sell 30.0 million Reais with expiration dates ranging from April 2016 through February 2019. These hedges were entered into in order to protect against the fluctuations in revenue and operating expenses associated with foreign currency-denominated cash flows. 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 and operating expenses denominated in currencies other than the U.S. dollar 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 selling, general and administrative (SG&A) expense in the Company's Condensed Consolidated Statements of Comprehensive Loss. At March 31, 2016, the Company had 17 outstanding forward foreign currency exchange contracts to sell 73.6 million Euros, 22 outstanding forward foreign currency contracts to purchase 55.9 million Euros and one outstanding forward foreign currency exchange contract to sell 5.4 million British Pounds, which were not designated as hedges for accounting purposes, with expiration dates ranging from April 2016 through July 2018.

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 February 2019. Over the next twelve months, the Company expects to reclassify \$1.1 million from accumulated other comprehensive income to earnings as the forecasted revenue and operating expense 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, 2016		Liability Derivatives March 31, 2016	
	Balance Sheet Location	Fair Value	Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ 6,912	Accounts payable and accrued liabilities	\$ 5,993
Forward foreign currency exchange contracts	Other assets	734	Other long- term liabilities	4,004
Total		7,646		9,997
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	46	Accounts payable and accrued liabilities	1,007
	Other assets	—	Other long- term liabilities	—
Total		46		1,007
Total value of derivative contracts		\$ 7,692		\$ 11,004

	Asset Derivatives December 31, 2015		Liability Derivatives December 31, 2015	
	Balance Sheet Location	Fair Value	Balance Sheet Location	Fair Value
Derivatives designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	\$ 10,478	Accounts payable and accrued liabilities	\$ 1,986
Forward foreign currency exchange contracts	Other assets	3,533	Other long- term liabilities	3,057
Total		14,011		5,043
Derivatives not designated as hedging instruments:				
Forward foreign currency exchange contracts	Other current assets	—	Accounts payable and accrued liabilities	22
Total		—		22
Total value of derivative contracts		\$ 14,011		\$ 5,065

The effect of the Company's derivative instruments on the Condensed Consolidated Financial Statements for the three months ended March 31, 2016 and 2015 was as follows:

	Three Months Ended March 31,	
	2016	2015
<b>Derivatives Designated as Hedging Instruments:</b>		
Net gain (loss) recognized in Other Comprehensive Income (OCI) <sup>(1)</sup>	\$(8,481)	\$13,776
Net gain reclassified from accumulated OCI into earnings <sup>(2)</sup>	3,327	4,739
Net gain recognized in net loss <sup>(3)</sup>	4,663	141
<b>Derivatives Not Designated as Hedging Instruments:</b>		
Net gain (loss) recognized in net loss <sup>(4)</sup>	\$(2,260)	\$7,800

(1) Net change in the fair value of the effective portion classified as OCI.

(2) Effective portion classified as Net Product Revenues and SG&A expense.

(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, 2016 and December 31, 2015, accumulated other comprehensive income associated with forward foreign currency exchange contracts qualifying for hedge accounting treatment was a gain of \$2.5 million and a gain of \$13.6 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)

## (13) 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, 2016			
	Quoted Price in			
	Active			
	Markets	Significant Other	Significant	
For	Observable	Unobservable		
Identical	Inputs	Inputs		
Assets	(Level 1)	(Level 2)	(Level 3)	Total
<b>Assets:</b>				
<b>Cash and cash equivalents:</b>				
Overnight deposits	\$261,236	\$ —	\$ —	\$261,236
Money market instruments	—	9,217	—	9,217
<b>Total cash and cash equivalents</b>	<b>261,236</b>	<b>9,217</b>	<b>—</b>	<b>270,453</b>
<b>Available-for-sale securities:</b>				
<b>Short-term:</b>				
Certificates of deposit	—	5,585	—	5,585
Corporate debt securities	—	74,504	—	74,504
Commercial paper	—	6,247	—	6,247
U.S. government agency securities	—	100,064	—	100,064
<b>Long-term:</b>				
Certificates of deposit	—	5,932	—	5,932
Corporate debt securities	—	247,851	—	247,851
U.S. government agency securities	—	60,497	—	60,497
Greek government-issued bonds	—	124	—	124
<b>Total available-for-sale securities</b>	<b>—</b>	<b>500,804</b>	<b>—</b>	<b>500,804</b>
<b>Other current assets:</b>				
Nonqualified Deferred Compensation Plan assets	—	170	—	170
Forward foreign currency exchange contract <sup>(1)</sup>	—	6,958	—	6,958
Restricted investments <sup>(2)</sup>	—	7,350	—	7,350
<b>Total other current assets</b>	<b>—</b>	<b>14,478</b>	<b>—</b>	<b>14,478</b>
<b>Other assets:</b>				

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 10-Q

Nonqualified Deferred Compensation Plan assets	—	7,464	—	7,464
Forward foreign currency exchange contract <sup>(1)</sup>	—	734	—	734
Strategic investment <sup>(3)</sup>	8,142	—	—	8,142
Total other assets	8,142	8,198	—	16,340
Total assets	\$269,378	\$ 532,697	\$ —	\$802,075
<b>Liabilities:</b>				
<b>Current liabilities:</b>				
Nonqualified Deferred Compensation Plan liability	\$789	\$ 170	\$ —	\$959
Forward foreign currency exchange contract <sup>(1)</sup>	—	7,000	—	7,000
Contingent acquisition consideration payable	—	—	97,449	97,449
Total current liabilities	789	7,170	97,449	105,408
<b>Other long-term liabilities:</b>				
Nonqualified Deferred Compensation Plan liability	18,823	7,464	—	26,287
Forward foreign currency exchange contract <sup>(1)</sup>	—	4,004	—	4,004
Contingent acquisition consideration payable	—	—	135,275	135,275
Total other long-term liabilities	18,823	11,468	135,275	165,566
Total liabilities	\$19,612	\$ 18,638	\$ 232,724	\$270,974



## 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, 2015			
	Quoted Price in			
	Active	Significant Other	Significant	
	Markets			
	For	Observable	Unobservable	
	Identical	Inputs	Inputs	
	Assets			
	(Level 1)	(Level 2)	(Level 3)	Total
<b>Assets:</b>				
Cash and cash equivalents:				
Overnight deposits	\$ 290,731	\$ —	\$ —	\$ 290,731
Money market instruments	—	106,309	—	106,309
Total cash and cash equivalents	290,731	106,309	—	397,040
Available-for-sale securities:				
Short-term:				
Certificates of deposit	—	56,951	—	56,951
Corporate debt securities	—	42,673	—	42,673
Commercial paper	—	12,733	—	12,733
U.S. government agency securities	—	83,222	—	83,222
Long-term:				
Certificates of deposit	—	6,969	—	6,969
Corporate debt securities	—	315,240	—	315,240
U.S. government agency securities	—	103,316	—	103,316
Greek government-issued bonds	—	127	—	127
Total available-for-sale securities	—	621,231	—	621,231
Other current assets:				
Nonqualified Deferred Compensation Plan assets	—	440	—	440
Forward foreign currency exchange contract <sup>(1)</sup>	—	10,478	—	10,478
Restricted investments <sup>(2)</sup>	—	7,348	—	7,348
Total other current assets	—	18,266	—	18,266
Other assets:				
Nonqualified Deferred Compensation Plan assets	—	6,362	—	6,362
Forward foreign currency exchange contract <sup>(1)</sup>	—	3,533	—	3,533
Strategic investment <sup>(3)</sup>	18,056	—	—	18,056
Total other assets	18,056	9,895	—	27,951
Total assets	\$ 308,787	\$ 755,701	\$ —	\$ 1,064,488
<b>Liabilities:</b>				
Current liabilities:				
Nonqualified Deferred Compensation Plan liability	\$ 1,151	\$ 440	\$ —	\$ 1,591

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 10-Q

Forward foreign currency exchange contract <sup>(1)</sup>	—	2,008	—	2,008
Contingent acquisition consideration payable	—	—	52,946	52,946
Total current liabilities	1,151	2,448	52,946	56,545
Other long-term liabilities:				
Nonqualified Deferred Compensation Plan liability	24,341	6,362	—	30,703
Forward foreign currency exchange contract <sup>(1)</sup>	—	3,057	—	3,057
Contingent acquisition consideration payable	—	—	32,663	32,663
Total other long-term liabilities	24,341	9,419	32,663	66,423
Total liabilities	\$25,492	\$ 11,867	\$ 85,609	\$122,968

(1) See Note 12 to these Condensed Consolidated Financial Statements for further information regarding the derivative instruments.

(2) The restricted investments at March 31, 2016 and December 31, 2015 secure the Company's irrevocable standby letter of credit obtained in connection with certain commercial 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)

(3)The Company has investments in marketable equity securities measured using quoted prices in an active market that are considered strategic investments. See Note 7 to these Condensed Consolidated Financial Statements for additional discussion regarding the Company’s strategic investments.

There were no transfers between levels during the three months ended March 31, 2016.

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 7 to these Condensed Consolidated Financial Statements for further information regarding the Company’s financial instruments.

Liabilities measured at fair value using Level 3 inputs consisted 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 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, 2015	\$85,609
Addition of contingent acquisition consideration payable related to	
the purchase of the Merck PKU Business	138,974
Changes in the fair value of contingent acquisition	
consideration payable	2,936
Foreign exchange remeasurement of Euro denominated contingent	
acquisition consideration payable	5,205
Contingent acquisition consideration payable at March 31, 2016	\$232,724

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, 2015	\$4,704
Accretion expense	2
Reversals	(250 )
Asset retirement obligations at March 31, 2016	\$4,456

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)

## (14) 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 and the 2014 Inducement Plan expired in June 2015. The Company's stock-based compensation plans are administered by the Compensation Committee of the Company's Board of Directors (the Board), 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 awards. See Note 18 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2015 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, and as of March 31, 2016 the Company has identified two groups with distinctly different exercise patterns. The two groups identified are executive and non-executive employees. The executive employee group has a history of holding options for longer periods than non-executive employees. 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, the 2014 Inducement Plan and the Share Incentive Plan were as follows:

	Three Months Ended March 31,	
	2016	2015
Expected volatility	36 – 44%	44 – 45%
Dividend yield	0.0%	0.0%
Expected life	6.5 - 8.1 years	7.0 years
Risk-free interest rate	1.5 – 2.1%	1.5 – 2.0%

During the three months ended March 31, 2016, the Company granted options to purchase 715,540 shares of common stock with a weighted average fair value of \$41.67 per share.

The Company did not issue any new stock purchase rights under the ESPP during the three months ended March 31, 2016.

Restricted Stock Unit Awards with Service-Based Vesting Conditions

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 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, 2016, the Company granted 1,118,734 RSUs with a weighted average fair value of \$83.30 per share.

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

During 2012 and 2011, pursuant to the approval of 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. The 2011/2012 Base RSUs had a weighted-average grant date fair value of \$34.66 and vested on February 29, 2016, based upon the achievement of the Vimizim approval and the 2015 revenue goal. The Company determined the number of Base RSUs that were earned on the vesting date (the Earned RSUs) and to determine the total number of RSUs to be awarded the Earned RSUs were multiplied by the Total Shareholder Return multiplier of 124% resulting in the issuance of 799,800 shares on February 29, 2016. Stock-based compensation expense for this award was recognized over the service period beginning in the period the Company determined the achievement of the strategic performance goal or goals were probable.

## BIOMARIN PHARMACEUTICAL INC.

## NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## Restricted Stock Unit Awards with Performance Conditions

On March 15, 2016, pursuant to Board approval, the Company granted 130,310 RSU awards with performance-vesting conditions (the 2016 Base RSUs) under the Share Incentive Plan to certain executive officers. The vesting of the 2016 Base RSUs under this specific grant is contingent upon the achievement of a 2016 revenue target and a three-year service period. The number of RSUs to be awarded from the 2016 Base RSUs upon achievement of the performance condition shall be calculated by multiplying the 2016 Base RSUs by a revenue multiplier (determined based on the Company's performance against the revenue target), which could range between 80% to 120%.

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 2016 Base RSUs was determined to be \$83.43 per RSU, based on the fair value of the common stock underlying the 2016 Base RSUs on the grant date. The Company evaluated the 2016 revenue target in the context of its current 2016 revenue forecast and related confidence level in the forecast, and determined that attainment of the revenue target was probable for accounting purposes. As a result, the Company recognized \$0.2 million of compensation expense related to these awards during the three months ended March 31, 2016.

On March 3, 2015, pursuant to Board approval, the Company granted 58,300 RSU awards with performance-vesting conditions (the 2015 Base RSUs) and a three-year service period, under the Share Incentive Plan, to certain executive officers. Based on the Company's performance against the 2015 revenue target, the Company applied a multiplier of 111% and issued 64,713 RSU awards with a grant date fair value of \$108.36 per RSU. The Company recognized \$0.8 million and \$0.2 million of compensation expense related to these awards during the three months ended March 31, 2016 and 2015, respectively.

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,	
	2016	2015
Cost of sales	\$1,562	\$1,348
R&D	13,707	9,930
SG&A	14,908	11,414
Total stock-based compensation expense	\$30,177	\$22,692

Stock-based compensation of \$2.5 million was capitalized into inventory for each of the three months ended March 31, 2016 and 2015. Capitalized stock-based compensation is recognized as Cost of Sales when the related product is sold.

## (15) COMPREHENSIVE LOSS

The following table summarizes amounts reclassified out of Accumulated Other Comprehensive Income (AOCI) and their effect on the Company's Condensed Consolidated Statements of Comprehensive Loss for the three months ended March 31, 2016 and 2015.

Details about AOCI Components	Three Months Ended		Consolidated Statement of Comprehensive Income (Loss) Classification
	March 31, 2016	2015	
Gains on cash flow hedges:			
Forward foreign currency exchange			
contracts	\$3,189	\$4,739	Net product revenues
Forward foreign currency exchange			
contracts	4,432	—	SG&A
	\$7,621	\$4,739	Net loss



## 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 tables summarize changes in the accumulated balances for each component of AOCI, including current period other comprehensive income and reclassifications out of AOCI, net of tax, for the three months ended March 31, 2016 and 2015.

	Three Months Ended March 31, 2016			
	Losses on Cash Flow Hedges	Unrealized Gains on Available-for-Sale Securities	Foreign Currency Items	Total
AOCI balance at December 31, 2015	\$ 13,602	\$ 7,441	\$ (10 )	\$ 21,033
Other comprehensive income (loss) before reclassifications	(8,481 )	(7,679 )	3	(16,157)
Less net gain reclassified from AOCI	7,621	—	—	7,621
Tax effect	—	2,792	—	2,792
Net current-period other comprehensive income (loss)	(16,102)	(4,887 )	3	(20,986)
AOCI balance at March 31, 2016	\$(2,500 )	\$ 2,554	\$ (7 )	\$ 47

	Three Months Ended March 31, 2015			
	Losses on Cash Flow Hedges	Unrealized Gains on Available-for-Sale Securities	Foreign Currency Items	Total
AOCI balance at December 31, 2014	\$ 15,906	\$ 11,511	\$ 49	\$ 27,466
Other comprehensive income (loss) before reclassifications	13,776	11,481	(5 )	25,252
Less gain reclassified from AOCI	4,739	—	—	4,739
Tax effect	—	(4,160 )	—	(4,160 )
Net current-period other comprehensive income (loss)	9,037	7,321	(5 )	16,353
AOCI balance at March 31, 2015	\$ 24,943	\$ 18,832	\$ 44	\$ 43,819

## (16) REVENUE AND CREDIT CONCENTRATIONS

Net Product Revenue—The Company considers there to be revenue concentration risks for regions where net product revenue exceeds 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 which are sold directly by the Company and global sales of Aldurazyme which is marketed by Genzyme Corporation (Genzyme). 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 consisted of royalties on worldwide net Aldurazyme sales and incremental product transfer revenues.

	Three Months Ended March 31, 2016 2015	
Net product revenues marketed by the Company		
United States	39 %	35 %
Europe	27 %	17 %
Latin America	8 %	21 %
Rest of world	19 %	18 %
Total net product revenue marketed by the Company	93 %	91 %
Aldurazyme net product revenues marketed by Genzyme	7 %	9 %
Total net product revenues	100%	100 %

## 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 illustrates the percentage of the Company's consolidated net product revenues attributed to the Company's five largest customers for the three months ended March 31, 2016 and 2015.

	Three Months Ended March 31, 2016 2015	
Customer A	20 %	15 %
Customer B <sup>(1)</sup>	7 %	9 %
Customer C	—	11 %
Customer D	14 %	12 %
Customer E	10 %	—
Total	51 %	47 %

(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 consisted of royalties on worldwide net Aldurazyme sales and incremental product transfer revenues.

On a consolidated basis, the Company's two largest customers accounted for 28% and 24% of the March 31, 2016 accounts receivable balance, respectively, compared to December 31, 2015, when the two largest customers accounted for 37% and 18% of the accounts receivable balance. As of March 31, 2016, and December 31, 2015, the accounts receivable balance for the Company's largest customer included \$29.9 million and \$36.1 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. For the three months ended March 31, 2016, the Company's net product revenues for these countries was 8%. Additionally, approximately 14% of the Company's outstanding accounts receivable at March 31, 2016 related to such countries.

As of March 31, 2016, the Company's accounts receivable in certain European countries, specifically Italy, Portugal, Spain and Russia, totaled approximately \$25.0 million, of which \$1.2 million were greater than 90 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.

## 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) 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 the Company's 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,	
	2016	2015
Net product revenues by product:		
Vimizim	\$72,578	\$50,622
Naglazyme	65,403	78,167
Kuvan	76,692	50,193
Aldurazyme	16,445	18,243
Firdapse	4,239	4,087
Total net product revenues	\$235,357	\$201,312

Net product revenues are 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,	
	2016	2015
Total revenues by geographic region:		
United States	\$109,503	\$90,248
Europe	63,283	41,710
Latin America	18,379	34,818
Rest of world	45,571	36,144
Total revenues	\$236,736	\$202,920

(18) 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.

25

---

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

Paragraph IV Notices

The Company received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying it that Par had 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 Food and Drug Administration's (FDA) Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Together with Merck & Cie, on March 6, 2015, the Company filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of its patents relating to Kuvan tablets and seeking an injunction to prevent Par from introducing a generic version of Kuvan tablets that would infringe its patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2017. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

The Court has set a claim construction hearing for May 5, 2016. The Court has not yet set a date for trial in this litigation.

The Company also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying the Company that Par has filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder prior to the expiration of the Company's patents listed in the FDA's Orange Book. On February 22, 2016, the Company filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of its patents relating to Kuvan powder and seeking an injunction to prevent Par from introducing a generic version of Kuvan powder that would infringe its patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2018. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid. The Court has set a scheduling conference for May 4, 2016.

Contingent Payments

As of March 31, 2016, the Company is subject to contingent payments totaling approximately \$880.8 million upon achievement of certain regulatory and licensing milestones if they occur before certain dates in the future. Of this amount, \$209.7 million (or €185 million based on the exchange rate of 1.13 USD per Euro in effect on March 31, 2016) relates to the Merck PKU Business acquisition, \$80.0 million relates to the Company's acquisition of Prosensa Holding N.V. (Prosensa) acquisition, and \$23.9 million relates to programs that are no longer being developed.

As of March 31, 2016, the Company has recorded \$232.7 million of contingent acquisition consideration payable on its Condensed Consolidated Balances Sheets in Short-term and Long-term Contingent Acquisition Consideration Payable, of which \$97.4 million is expected to be paid in the next twelve months.

Other Commitments

In the normal course of business, the Company enters into various firm purchase commitments primarily related to active pharmaceutical ingredients and certain inventory related items. As of March 31, 2016, these commitments for the next five years were approximately \$52.5 million. The amounts primarily represent minimum purchase

requirements for active pharmaceutical ingredients and post-marketing commitments related to the Company's approved products.

(19) 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 has computed the U.S. component of the consolidated provision for (benefit from) income taxes for the three months ended March 31, 2016 and 2015 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, 2016 and 2015.



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," "intends," "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 the part of this Item 2 entitled "Overview" 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, 2015, which was filed with the Securities and Exchange Commission (the SEC) on February 29, 2016. 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 consisted of five approved products and multiple clinical and pre-clinical product candidates. Our approved products are Vimizim (elosulfase alfa), Naglazyme (galsulfase), Kuvan (sapropterin dihydrochloride), Aldurazyme (laronidase) and Firdapse (amifampridine phosphate).

#### Business Developments

During the three months ended March 31, 2016, we continued to grow our commercial business and advance our product pipeline. We believe that the combination of our internal research programs, acquisitions and partnerships will allow us to continue develop and commercialize innovative biopharmaceuticals for serious diseases and medical conditions. Below is a summary of key business developments in this quarter:

- In January 2016, we acquired all global rights, with the exception of Japan, to Kuvan and pegvaliase (collectively, the Merck PKU Business) from Ares Trading S.A. (Merck Serono), an indirectly wholly-owned affiliate of Merck KGaA, in exchange for cash payments of \$374.2 million. We also agreed to pay Merck Serono up to a maximum of €60.0 million in milestones if certain sales milestones are met and up to a maximum of €125.0 million if certain pegvaliase development milestones are met. See Note 5 to our accompanying Condensed Consolidated Financial Statements for additional discussion.
- We received Orphan Drug Designation for the first AAV-Factor VIII Gene Therapy, BMN 270, for patients with Hemophilia A from the Federal Drug Administration (FDA) and the European Commission (EC).

- In April 2016, we announced preliminary data on the first eight patients from our ongoing Phase 1/2 trial of BMN 270, which showed that all high dose patients improved from severe hemophilia to either moderate, mild or normal range in terms of factor levels based on World Federation of Hemophilia criteria.
- We announced that our pivotal Phase 3 PRISM-2 study of pegvaliase met the primary endpoint of change in blood Phe compared with placebo ( $p < 0.0001$ ) in preliminary results.
- We announced positive 48-week results from our Phase 1/2 pivotal study of cerliponase alfa for treatment of children with CLN2 Disease, a form of Batten disease.

27

---

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

- We announced enrollment of the first patient in a Phase 1/2 trial for BMN 250, an investigational enzyme replacement therapy using a novel fusion of recombinant human alpha-N-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome or mucopolysaccharidosis IIIB (MPS IIIB).
- In January 2016 the Food and Drug Administration (FDA) issued a complete response letter to our New Drug Application for Kyndrisa for the treatment of Duchenne muscular dystrophy amenable to exon 51 skipping. The FDA concluded that the standard of substantial evidence of effectiveness for Kyndrisa had not been met. Based on the current U.S. development efforts, we recognized an impairment of the Kyndrisa IPR&D assets totaling \$198.7 million in the fourth quarter of 2015.
  - We reported total revenues of \$236.7 million for the three months ended March 31, 2016 as compared to \$202.9 million for the three months ended March 31, 2015.

## Financial Highlights

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

	Three Months Ended March 31,	
	2016	2015
Total revenues	\$236.7	\$202.9
Cost of sales	43.1	31.0
Research & Development (R&D) expense	158.8	142.1
Selling, general and administrative (SG&A) expense	105.3	92.8
Intangible asset amortization and contingent consideration expense	10.4	2.9
Net loss	(85.1 )	(67.5 )
Stock-based compensation expense	30.2	22.7

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,	
	2016	2015
Vimizim	\$72.6	\$50.6
Naglazyme	65.4	78.2
Kuvan	76.7	50.2
Aldurazyme	16.4	18.2
Firdapse	4.3	4.1
Total net product revenues	\$235.4	\$201.3

Cost of sales includes raw materials, personnel and facility and other costs associated with manufacturing Vimizim, Naglazyme and Aldurazyme at our production facilities. 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.

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

Our cash, cash equivalents, short-term investments and long-term investments totaled \$771.3 million as of March 31, 2016, compared to \$1,018.3 million as of December 31, 2015. 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 revenues 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. 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 United States (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 the three months ended March 31, 2016, 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, 2015.

#### Recent Accounting Pronouncements

See Note 4 to our accompanying Condensed Consolidated Financial Statements for a 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, 2016, was \$85.1 million, compared to a net loss of \$67.5 million for the three months ended March 31, 2015. The increase in net loss was primarily a result of the following (in millions):

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 10-Q

	Three Months Ended		
	March 31,		
	2016	2015	Change
Total revenues	\$236.7	\$202.9	\$ 33.8
Cost of Sales	43.1	31.0	12.1
R&D expense	158.8	142.1	16.7
SG&A expense	105.3	92.8	12.5
Intangible asset amortization and contingent consideration	10.4	2.9	7.5
Other, net	(8.2 )	(8.8 )	0.6
Provision for (benefit from) income taxes	(4.0 )	(7.2 )	3.2
Net loss	\$(85.1 )	\$(67.5 )	\$(17.6 )

29

---

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

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

Net Product Revenues, Cost of Sales and Gross Profit

A summary of our various commercial products, including key metrics as of March 31, 2016, is provided below:

Commercial Products	Indication	Orphan Drug Exclusivity Expiration U.S.	Orphan Drug Exclusivity Expiration EU
Vimizim	MPS IVA <sup>(1)</sup>	2021	2024
Naglazyme	MPS VI <sup>(2)</sup>	Expired	Expired
Kuvan	PKU <sup>(3)</sup>	Expired	2020
Aldurazyme <sup>(4)</sup>	MPS I <sup>(5)</sup>	Expired	Expired
Firdapse	LEMS <sup>(6)</sup>	NA <sup>(8)</sup>	2019

(1) Mucopolysaccharidosis IV Type A (MPS IVA)

(2) Mucopolysaccharidosis VI (MPS VI)

(3) Phenylketonuria (PKU)

(4) The Aldurazyme total product revenue noted above is the total product revenue recognized by us in accordance with the terms of our agreement with Genzyme Corporation (Genzyme).

(5) Mucopolysaccharidosis I (MPS I)

(6) Lambert Eaton Myasthenic Syndrome (LEMS)

(7) Firdapse has not received marketing approval in the U.S. and we have licensed the North American rights to develop and market Firdapse to a third party.

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

	Three Months Ended		
	March 31,		
	2016	2015	Change
Vimizim	\$72.6	\$50.6	\$22.0
Naglazyme	65.4	78.2	(12.8 )
Kuvan	76.7	50.2	26.5
Aldurazyme	16.4	18.2	(1.8 )
Firdapse	4.3	4.1	0.2
Total net product revenues	\$235.4	\$201.3	\$34.1

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

	Three Months Ended		
	March 31,		
	2016	2015	Change
Aldurazyme revenue reported by Genzyme	\$52.8	\$53.4	\$ (0.6 )

Edgar Filing: BIOMARIN PHARMACEUTICAL INC - Form 10-Q

Royalties earned from Genzyme Incremental (previously recognized)	\$21.5	\$22.3	\$ (0.8 )
Aldurazyme product transfer revenue	(5.1 )	(4.1 )	(1.0 )
Total Aldurazyme net product revenues	\$16.4	\$18.2	\$ (1.8 )

---

30



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

The FDA and the European Medicines Agency (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. Vimizim net product revenues for the three months ended March 31, 2016 totaled \$72.6 million, compared to \$50.6 million for the three months ended March 31, 2015. Vimizim net product revenues earned from customers based outside the U.S. during the three months ended March 31, 2016, totaled \$49.6 million, compared to \$34.6 million during the three months ended March 31, 2015. The increase in Vimizim net product revenues for the three months ended March 31, 2016 was attributable to new patients initiating therapy. The impact of foreign currency exchange rates on Vimizim sales was negative by \$1.8 million and \$4.3 million for the three months ended March 31, 2016 and 2015, respectively. Gross margin (net product revenues less costs of sales, expressed as a percentage of net product revenues) for Vimizim was 85% for the three months ended March 31, 2016 compared to gross margin of 86% for the three months ended March 31, 2015. In future periods, we do not expect Vimizim gross margins to fluctuate significantly.

Naglazyme net product revenues for the three months ended March 31, 2016 totaled \$65.4 million compared to \$78.2 million for the three months ended March 31, 2015. Naglazyme net product revenues earned from customers based outside the U.S. totaled \$54.0 million for the three months ended March 31, 2016, compared to \$68.5 million for the three months ended March 31, 2015. The decrease in Naglazyme net product revenues was attributable to the negative impact of foreign currency exchange rates and delays in purchases from certain government entities, offset by new patients initiating therapy. The impact of foreign currency exchange rates on Naglazyme during 2015 was negative by \$0.8 million for the three months ended March 31, 2016, compared to a negative impact of \$3.3 million for the three months ended March 31, 2015. Our gross margin for Naglazyme was 85% for the three months ended March 31, 2016, compared to gross margin of 87% for the three months ended March 31, 2015. In future periods, we do not expect Naglazyme gross margins to fluctuate significantly.

Kuvan net product revenues for the three months ended March 31, 2016 totaled \$76.7 million compared to \$50.2 million for the three months ended March 31, 2015. Kuvan net product revenues earned from customers based outside North America totaled \$16.6 million for the three months ended March 31, 2016. The increase in Kuvan net product revenues was attributable to international Kuvan product sales and new patients initiating therapy. Cost of goods sold for the three months ended March 31, 2016 and 2015 reflect royalties paid to third parties. Our gross margin for Kuvan was 76% for the three months ended March 31, 2016, compared to 83% for the three months ended March 31, 2015. During the first quarter of 2016, the cost of inventory acquired in the Merck PKU Business acquisition included a fair value adjustment, reducing gross margin for those units to a reasonable seller's profit. We expect the Kuvan inventory purchased from Merck Serono to be depleted in the second quarter of 2016 and Kuvan gross margins are expected to normalize to the mid-eighties in the second half of 2016. Prior to our acquisition of the Merck PKU Business, we earned royalty on Merck Serono's net sales of Kuvan of 4%.

In 2016, we expect Kuvan net product revenues to increase over 2015 levels as a result of our acquisition of the Merck PKU Business, which was effective January 1, 2016. Kuvan gross margins are not expected to fluctuate significantly in the future. However we expect to see generic competition for Kuvan in the future.

In September 2015, we entered into a settlement agreement with Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (DRL) that resolved patent litigation with DRL in the U.S. related to its abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan 100 mg oral tablets. Under the terms of the settlement agreement, we have granted DRL a non-exclusive license to our Kuvan related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than five years from the settlement date, or earlier under certain circumstances. The settlement does not affect the case against Par Pharmaceutical, Inc. (Par) with

respect to its ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Par has also filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder.

Our settlement with DRL and the filing of Par's purported ANDAs with respect to Kuvan 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 and its use are not upheld in litigation, or if 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. Moreover, generic competition from DRL following the settlement described above could have a material adverse effect on our revenue and results of operations.

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

Aldurazyme net product revenues for the three months ended March 31, 2016 totaled \$16.4 million compared to \$18.2 million for the three months ended March 31, 2015. The decrease in Aldurazyme net product revenues was primarily attributable to a decrease in Genzyme-reported Aldurazyme sales. Our gross margin on Aldurazyme was 81% for the three months ended March 31, 2016, compared to gross margin of 79% for the three months ended March 31, 2015. 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.

## Cost of Sales

Total cost of sales for the three months ended March 31, 2016 was \$43.1 million compared to \$31.0 million for the three months ended March 31, 2015. The increase in cost of sales was primarily attributable to the increase in product sales as well as recognition of the fair value adjustment to inventory acquired in the Merck PKU Business acquisition that was sold in the first quarter of 2016.

## Research and Development

A summary of our various major development programs, including key metrics as of March 31, 2016, is provided below:

Major Products in Development	Target Indication	U.S. Orphan Designation	EU Orphan Designation	Stage
Kyndrisa	DMD <sup>(1)</sup>	Yes	Yes	Clinical Phase 3
Pegvaliase	PKU	Yes	Yes	Clinical Phase 3
Reveglucosidase alfa	Pompe <sup>(2)</sup>	Yes	Yes	Clinical Phase 2/3
Vosoritide	Achondroplasia	Yes	Yes	Clinical Phase 2
Cerliponase alfa	CLN2 <sup>(3)</sup>	Yes	Yes	Clinical Phase 1/2
BMN 270	Hemophilia A	Yes	Yes	Clinical Phase 1/2

(1) Duchenne muscular dystrophy (DMD). Kyndrisa was acquired from Prosensa Holding N.V. (Prosensa) in January 2015

(2) Pompe disease

(3) CLN2, or late infantile neuronal ceroid lipofuscinosis, is a lysosomal storage disorder primarily affecting the brain

(4) BMN 270 is an investigational gene therapy for Hemophilia A, also called factor VIII deficiency or classic hemophilia

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 \$158.8 million for the three months ended March 31, 2016, from \$142.1 million for the three months ended March 31, 2015. R&D expense consisted of the following (in millions):

	Three Months Ended		
	March 31,		
	2016	2015	Change
Pegvaliase	\$19.0	\$16.7	\$ 2.3
Kyndrisa	16.4	6.8	9.6
Cerliponase alfa	16.0	9.2	6.8
Reveglucosidase alfa	15.2	16.6	(1.4 )
Vosoritide	13.1	8.7	4.4
Vimizim	7.8	12.5	(4.7 )
Talazoparib <sup>(1)</sup>	2.1	18.3	(16.2 )
Other approved products	10.2	8.1	2.1
Early stage programs	28.1	13.2	14.9
Other and non-allocated	30.9	32.0	(1.1 )
<b>Total</b>	<b>\$158.8</b>	<b>\$142.1</b>	<b>\$ 16.7</b>

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

(1) In October 2015, we sold talazoparib to Medivation. For the three months ended March 31, 2016, talazoparib R&D expense primarily related to employee-related wind-down costs.

The increase in pegvaliase, cerliponase alfa, and vosoritide expense was attributable to increased clinical trial activities related to these product candidates. The development expenses for Kyndrisa relate to clinical and European regulatory activities for this product candidate. The increase in development expense related to early development stage programs was primarily attributable to the pre-clinical activity related to BMN 270 and NAGLU. The decrease in talazoparib expenses was due to the completion of the sale to Medivation in the fourth quarter of 2015.

During the remainder of 2016, we expect our R&D spending to increase over 2015 levels due to our Kyndrisa, pegvaliase, reveglucosidase alfa, vosoritide and cerliponase alfa programs progressing in their development. We also expect increased spending on pre-clinical and clinical activities for our early development stage programs, including BMN 270 and NAGLU, and other pre-clinical programs. 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.

## Selling, General and Administrative

SG&A expense increased to \$105.3 million for the three months ended March 31, 2016 from \$92.8 million for the three months ended March 31, 2015. The increase in SG&A expense was primarily a result of the following (in millions):

	Three Months Ended March 31,		
	2016	2015	Change
Sales and marketing (S&M) expense	\$53.7	\$41.7	\$ 12.0
General and administrative (G&A) expense	51.6	51.1	0.5
Total SG&A expense	\$105.3	\$92.8	\$ 12.5

	Three Months Ended March 31,		
S&M expense by product	2016	2015	Change
Vimizim	\$14.3	\$13.3	\$ 1.0
Naglazyme	11.5	10.6	0.9
Kuvan	14.5	10.0	4.5
Other and not allocated	13.4	7.8	5.6
Total S&M expense	\$53.7	\$41.7	\$ 12.0

S&M expense primarily consists of employee-related expenses for our sales group, brand marketing, patient support groups and pre-commercialization expenses related to our product candidates. We re-acquired the worldwide rights, except for Japan, for Kuvan in January 2016. The increase in Kuvan S&M expense is attributable to this acquisition. We continue to incur S&M expense for Naglazyme and Vimizim as a result of continued expansion of our

international and worldwide activities, respectively. The increase in other S&M expense was driven by an increase in the number of sales and marketing employees and pre-commercialization marketing expense for Kyndrisa and cerliponase alfa.

G&A primarily consists of corporate support and other administrative expenses, which increased primarily due to increased employee-related expenses, consulting fees, and information technology expenses, offset by decreased business acquisition costs.

We expect SG&A expense to increase in future periods as a result of pre-commercialization expense related to product candidates, the international expansion of Naglazyme, Vimizim and Kuvan, and the increase in administrative support required for our expanding operations.

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

## Intangible Asset Amortization and Contingent Consideration

Intangible asset amortization and contingent consideration expense consists of changes in the fair value of contingent acquisition consideration payable in respect 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,		
	2016	2015	Change
Changes in the fair value of contingent			
acquisition consideration payable	\$2.9	\$0.3	\$ 2.6
Amortization of intangible assets	7.5	2.6	4.9
Total intangible asset amortization and			
contingent consideration	\$10.4	\$2.9	\$ 7.5

The changes in the fair value of the contingent acquisition consideration payable were primarily attributable to changes in the estimated probability of achieving development milestones based on the current status of the related development programs as well as the passage of time. During the three months ended March 31, 2016, the majority of the increase in the fair value of the contingent acquisition consideration payable was attributed to development progress of pegvaliase, for which contingent consideration expense was \$2.9 million. The increase in amortization of intangible assets was primarily attributable to the amortization of the Kuvan intangible asset acquired from Merck Serono in January 2016.

## 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 \$1.6 million for the three months ended March 31, 2016, compared to \$0.7 million for the three months ended March 31, 2015. We do not expect future interest income to fluctuate significantly over the next twelve months.

## Interest Expense

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

	Three Months Ended March 31,		
	2016	2015	Change
Coupon interest	\$2.5	\$2.5	\$ —
Amortization of issuance costs	0.8	0.8	—

Accretion of discount on convertible notes	6.5	6.2	0.3
Total interest expense	\$9.8	\$9.5	\$ 0.3

Interest expense consisted of 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 11 to our accompanying Condensed Consolidated Financial Statements for additional information regarding our Convertible Debt.

#### Benefit from Income Taxes

For the three months ended March 31, 2016, we recognized a benefit from income taxes of \$4.0 million compared to the three months ended March 31, 2015 when we recognized a benefit from income taxes of \$7.2 million. We have historically computed our 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 used in computing the effective tax rate, the effective tax rate is highly sensitive to minor fluctuations in U.S. forecasted income. As such, we have computed the U.S. component of the consolidated provision for (benefit from) income taxes for the three months ended March 31, 2016 and 2015 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, 2016 and 2015.



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

The benefit from income taxes for the three months ended March 31, 2016 and 2015 consisted of state, federal and foreign current tax expense that was offset by deferred tax benefits from federal orphan drug and the federal and California R&D credits, and the tax benefit related to stock option exercises during these periods. See Note 16 to our Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2015 for additional discussion of the components of our provision for (benefit from) income taxes.

## 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 revenues 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.

We consider the unrepatriated cumulative earnings of certain of our foreign subsidiaries to be indefinitely invested outside the U.S. As of March 31, 2016, \$151.2 million of our \$771.3 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. For additional discussion, see Note 16 to our Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2015.

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 of uncertainty with respect to, 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 liquidity and capital resources as of March 31, 2016 and December 31, 2015 were as follows (in millions):

	March 31, 2016	December 31, 2015	Change
Cash and cash equivalents	\$ 270.5	\$ 397.0	\$(126.5)
Short-term investments	186.4	195.6	(9.2 )
Long-term investments	314.4	425.7	(111.3)
Cash, cash equivalents and investments	\$ 771.3	\$ 1,018.3	\$(247.0)
Current assets	\$ 992.8	\$ 1,089.6	\$(96.8 )
Current liabilities	388.0	445.5	(57.5 )
Working capital	\$ 604.8	\$ 644.1	\$(39.3 )
Convertible debt	\$ 668.0	\$ 662.3	\$5.7

Our cash flows for the three months ended March 31, 2016 and 2015 are summarized as follows (in millions):

	2016	2015	Change
Cash and cash equivalents at the beginning of the period	\$397.0	\$875.5	\$(478.5)
Net cash used in operating activities	(171.6)	(137.8 )	(33.8 )
Net cash provided by (used in) investing activities	77.0	(750.9 )	827.9
Net cash provided by (used in) financing activities	(37.2 )	914.8	(952.0)
Foreign exchange impact	5.3	(1.0 )	6.3
Cash and cash equivalents at the end of the period	\$270.5	\$900.6	\$(630.1)
Short-term and long-term investments	500.8	332.0	168.8
Cash, cash equivalents and investments	\$771.3	\$1,232.6	\$(461.3)

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

## Working Capital

Working capital decreased by \$39.3 million, from \$644.1 million at December 31, 2015 to \$604.8 million at March 31, 2016. The decrease in working capital was attributed to the following (in millions):

Working capital at December 31, 2015	\$644.1
Decreased cash, cash equivalents and short-term investments	(135.7)
Increased accounts receivable, net	15.8
Increased inventory	25.3
Decreased current liabilities	57.4
Decreased other current assets	(2.1 )
Working capital at March 31, 2016	\$604.8

The decrease in cash, cash equivalents and short-term investments was primarily attributed to \$171.6 million of cash used to fund operating activities, \$45.2 million net cash invested in property, plant, and equipment, and \$40.8 million for taxes paid related to net share settlement of equity awards, partially offset by the net maturities of \$122.4 million of available-for-sale investments. The increase in accounts receivable was attributed to increased revenues and the timing of cash receipts from customers. The increase in inventory was primarily attributed to the manufacture of inventories for all commercial products to meet anticipated future sales demand. The decrease in current liabilities was primarily due to a \$101.9 million decrease in accounts payable and accrued expenses related to payments of R&D expenses and income taxes, offset by an increase of \$44.5 million in short-term contingent acquisition consideration payable related to the Merck PKU Business acquisition.

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 authorities. 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, 2016, approximately 14% of our outstanding accounts receivable relate to such countries. See Note 16 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, 2016 was \$171.6 million, compared to cash used in operating activities of \$137.8 million for the three months ended March 31, 2015. Cash used in operating activities primarily consisted of net loss of \$85.1 million, adjusted for non-cash items such as \$30.2 million for stock-based compensation expenses, \$20.2 million for depreciation and amortization expense, \$7.3 million of non-cash interest expense, offset by \$19.3 million for deferred income taxes benefit. Changes in operating assets and

liabilities resulted in a net cash outflow of \$122.0 million that consisted primarily of increased payments of R&D expenses and increased inventory purchases for all commercial products to meet anticipated future sales demand.

#### Cash Provided By (Used in) Investing Activities

Net cash provided by investing activities during the three months ended March 31, 2016 was \$77.0 million, compared to net cash used in investing activities during the three months ended March 31, 2015 of \$750.9 million. The increase in net cash provided by investing activities for the three months ended March 31, 2016 compared to the three months ended March 31, 2015, primarily consisted of a \$286.6 million increase in net purchases of available-for-sale securities and a \$538.4 million decrease in cash used to acquire a business. During 2016, we expect to continue to make significant capital investments in our manufacturing facilities and our corporate headquarters to accommodate anticipated headcount growth.

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

Cash Provided by (Used in) Financing Activities

Net cash used in financing activities for the three months ended March 31, 2016 was \$37.2 million, compared to net cash provided by financing activities of \$914.8 million for the three months ended March 31, 2015. The increase in net cash used in financing activities for the three months ended March 31, 2016, was primarily attributable to an \$888.3 million decrease in net proceeds from the public offering of common stock and a \$40.1 million increase in taxes paid related to net share settlement of equity awards.

Other Information

Our \$779.8 million (undiscounted) of total convertible debt as of March 31, 2016, 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. See Note 11 to our accompanying Condensed Consolidated Financial Statements for additional discussion.

On January 1, 2016, we acquired all global rights, with the exception of Japan, to Kuvan and pegvaliase (collectively, the Merck PKU Business) from Ares Trading S.A. (Merck Serono), an indirectly wholly-owned affiliate of Merck KGaA, Darmstadt, Germany, in exchange for cash payments of \$374.2 million, and up to €60.0 million in cash if future Kuvan sales milestones are met, and up to €125.0 million in cash if future pegvaliase development milestones are met.

On October 6, 2015, we completed the sale of talazoparib to Medivation, under which Medivation acquired the worldwide rights to talazoparib in exchange for payment of \$410.0 million and up to an additional \$160.0 million upon the achievement of regulatory and sales-based milestones and mid-single digit percentage royalties for talazoparib.

On January 27, 2015, we sold 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 accounting for the underwriting discount and offering costs.

On January 15, 2015, we closed the initial offering period related to our offer to purchase all of the ordinary shares of Prosensa (Prosensa Shares), 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 \$620.7 million for approximately 35 million Prosensa Shares, representing 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 was liquidated in January 2016 under Dutch law.

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 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.

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

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 the period since inception of our major development programs were as follows (in millions):

	Since Program Inception
Vimizim	\$ 410.9
Reveglucosidase alfa	222.1
Vosoritide	131.9
Cerliponase alfa	127.0
Pegvaliase	331.2
Kyndrisa	66.3
Other approved products	633.5
Other and non-allocated	Not meaningful

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;
- manufacturing, supply or distribution of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- progress of our integration of the PKU franchise rights acquired from Merck Serono;
- 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 lawsuits against Par to protect our patents relating to Kuvan and generic competition to Kuvan relating to our settlement with DRL;
- 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;
- changes in company assessments or financial estimates by securities analysts; and
- sales of our shares of stock by us, our significant stockholders, or members of our management or Board of Directors.

#### 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.





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

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. We are also subject to contingent payments related to various development activities totaling approximately \$880.8 million as of March 31, 2016, which are due upon achievement of certain development and commercial milestones, and if they occur before certain dates in the future. Of this amount, \$209.7 million (USD equivalent of 185 million Euros translated at 1.13 USD per Euro) relates to the Merck PKU Business acquisition, \$80.0 million relates to the Prosensa Holding N.V. acquisition, and \$23.9 million relates to programs that are no longer being developed.

There have been no material changes to the Company's contractual and commercial obligations during the three months ended March 31, 2016, as compared to the significant accounting policies disclosed in Management's Discussion and Analysis in the Company's Annual Report on Form 10-K for the year ended December 31, 2015.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Our market risks during the three months ended March 31, 2016 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, 2015, which was filed with the SEC on February 29, 2016.

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 were effective.

(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

We received a paragraph IV notice letter, dated January 22, 2015, from Par, notifying us that Par had filed an ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Together with Merck & Cie, on March 6, 2015, we filed a lawsuit against Par in the United States District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan tablets and seeking an injunction to prevent Par from introducing a generic version of Kuvan tablets that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2017. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

Court has set a claim construction hearing for May 5, 2016. The Court has not yet set a date for trial in this litigation.

We also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying us that Par has filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder prior to the expiration of our patents listed in the FDA's Orange Book. On February 22, 2016, we filed a lawsuit against Par in the United States District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan powder and

seeking an injunction to prevent Par from introducing a generic version of Kuvan powder that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2018. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid. The Court has set a scheduling conference for May 4, 2016.

#### 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, 2015, which was filed with the SEC on February 29, 2016.

#### Risks Related to Our Business

If we fail to obtain 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. For example, in January 2016, the FDA issued a complete response letter to our New Drug Application for Kyndrisa for the treatment of DMD amenable to exon 51 skipping in which the FDA concluded that the standard of substantial evidence of Kyndrisa's effectiveness had not been met. 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. A marketing authorization application (MAA) for Kyndrisa for the treatment of DMD amenable to exon 51 skipping remains under review in the EU. We anticipate that the Committee for Medicinal Products for Human Use (CHMP) of the EMA will provide an opinion for our MAA for Kyndrisa in the second quarter of 2016. If the CHMP opinion is positive, the MAA will be referred to the EC. The EC is expected to render a final decision for Kyndrisa in the second half of 2016. If the MAA is not approved by the EC, we will not receive marketing authorization for Kyndrisa in the EU and will be unable to sell Kyndrisa in the EU or roll out our international registration strategy for Kyndrisa using the EU approval as the basis of multiple international applications.

Although the FDA and the EMA have programs to facilitate accelerated approval processes, the timelines agreed under legislative goals or mandated by regulations are subject to the possibility of substantial delays. In addition, the FDA, the EMA and other international regulatory authorities have substantial discretion over the approval process for pharmaceutical products. These regulatory agencies may 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 foreign 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.

In addition, some of our product candidates, including cerliponase alfa, are intended to be used in combination with a delivery device, such as an injector or other delivery system. Medical products containing a combination of new drugs, biological products or medical devices may be regulated as "combination products" in the U.S. A combination product generally is defined as a product consisting of components from two or more regulatory categories (e.g., drug/device, device/biologic, drug/biologic). Each component of a combination product is subject to the requirements established by the FDA for that type of component, whether a new drug, biologic or device. In order to facilitate pre-market review of combination products, the FDA designates one of its centers to have primary jurisdiction for the pre-market review and regulation of the overall product based upon a determination by the FDA of the primary mode of action of the combination product. The determination whether a product is a combination product or two separate products is made by the FDA on a case-by-case basis. Our product candidates intended for use with such devices, or

expanded indications that we may seek for our products used with such devices, may not be approved or may be substantially delayed in receiving approval if the devices do not gain and/or maintain their own regulatory approvals or clearances. Where approval of the drug or biologic product and device is sought under a single application, the increased complexity of the review process may delay approval. The FDA review process and criteria is not a well-established area, which could also lead to delays in the approval process. In addition, because these delivery devices are provided by unaffiliated third-party companies, we are dependent on the sustained cooperation and effort of those third-party companies both to obtain regulatory approval and to maintain their own regulatory compliance. Failure of third-party companies to assist in the approval process or to maintain their own regulatory compliance could delay or prevent approval of our product candidates, or limit our ability to sell a product once it is approved.

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. 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.

Any product for which we have obtained regulatory approval, or for which we obtain approval in the future, is subject to, or will be subject to, extensive ongoing regulatory requirements by the FDA, the EMA and other comparable international regulatory authorities, and if we fail to comply with regulatory requirements or if we experience unanticipated problems with our approved products, we may be subject to penalties, we will be unable to generate revenue from the sale of such products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased.

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. Any product for which we have obtained regulatory approval, or for which we obtain regulatory approval in the future, along with the manufacturing processes and practices, post-approval clinical research, product labeling, advertising and promotional activities for such product, are subject to continual requirements of, and review by, the FDA, the EMA and other comparable international regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, current good manufacturing practices (cGMP) requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians, import and export requirements and recordkeeping.

Promotional communications with respect to prescription drugs, including biologics, are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we will not be able to promote any products we develop for indications or uses for which they are not approved. We also are subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMP and other regulations.

In addition, the FDA often requires post-marketing testing and surveillance to monitor the effects of approved products. The FDA, the EMA and other 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. Discovery after approval of previously unknown problems with any such products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in actions such as:

- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials;
  - restrictions on product manufacturing processes;
- restrictions on the marketing of a product;
- restrictions on product distribution;
  - requirements to conduct post-marketing clinical trials;
- untitled or warning letters or other adverse publicity;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- refusal to permit the import or export of our products;
- product seizure;
- fines, restitution or disgorgement of profits or revenue;
- injunctions; or
- imposition of civil or criminal penalties.

If such regulatory actions are taken, the value of our company and our operating results will be adversely affected. Additionally, if the FDA, the EMA or any other comparable international regulatory agency withdraws its approval of a product, we will be unable to generate revenue from the sale of that product in the relevant jurisdiction, our potential for generating positive cash flow will be diminished and the capital necessary to fund our operations will be increased. Accordingly, we continue to expend significant time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance, post-marketing studies and quality control.

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

As part of our business strategy, we have developed and may in the future 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. In the EU, orphan drug designation is granted to drugs intended to treat a rare disease or condition, defined as having a prevalence of no more than five in 10,000 people in the EU, which is equivalent to around 250,000 people or fewer. 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 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 of certain product candidates until the competitor 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 and the same drug can be approved for different conditions and potentially used off-label in the orphan indication. 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 or 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 Biologics Price Competition and Innovation Act of 2009 (BPCIA) created a regulatory pathway under the PHS Act for the abbreviated approval of 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 BPCIA 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. Aldurazyme's data exclusivity under the BPCIA expired in 2015, Naglazyme's data exclusivity under the BPCIA expires in 2017, and Vimizim's data exclusivity under the BPCIA expires in 2026. Our products approved under BLAs, as well as products in development that may be approved under BLAs in the future, could be reference products for biosimilar marketing applications.



To obtain regulatory approval to market our products, preclinical studies and costly and lengthy 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 for any of our product candidates 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 and experience net cash outflows 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 capital investments, and we have operated at a net loss for each year since our inception, with the exception of 2008 and 2010. Based upon our current plan for investments in research and development for existing and new programs, as well as capital investments in our facilities and working capital such as inventory, we expect to operate at a net loss and experience net cash outflows for at least the next 12 months. Our future profitability and cash flows depend 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, the impact of any possible future business development transactions and other risks set forth in this Risk Factors section. The extent of our future losses and the timing of profitability and positive cash flows are highly uncertain. If we fail to become profitable and cash flow positive or are unable to sustain profitability and positive cash flows 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 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, 2016, we had cash, cash equivalents and short and long-term investments totaling \$771.3 million and long-term debt obligations of \$779.8 million (undiscounted). In January 2016 we terminated our License and Commercialization Agreement with Ares Trading, S.A. (Merck Serono). Pursuant to the Termination and Transition Agreement related to Kuvan and the Termination Agreement related to pegvaliase, we made cash payments on this transaction totaling \$374.2 million, in December 2015, and may pay Merck Serono up to a maximum of €60 million, in

cash, if future sales milestones are met with respect to Kuvan and up to a maximum of €125 million, in cash, if future development milestones are met with respect to pegvaliase. In January 2015, we paid \$620.7 million for approximately 35 million ordinary shares (the Prosensa Shares) of Prosensa, 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 an additional \$80.0 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 (the 2018 Notes) and 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes and, together with the 2018 Notes, the Notes) but also the ongoing interest due on the Notes during their term. We will require additional financing to fund the repayment of our Notes, future milestone payments and 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 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 Therapeutics, Inc., Huxley Pharmaceuticals, Inc., and Zacharon Pharmaceuticals Inc., under the non-transferable CVRs issued in connection with the acquisition of Prosensa that trigger related milestone payments and under the termination agreements with Merck Serono related to Kuvan and pegvaliase milestones;
- 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 or arrangements.

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 will 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 will 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.

\*We have incurred substantial indebtedness that may decrease our business flexibility, access to capital, and/or increase our borrowing costs, which may adversely affect our operations and financial results.

As of March 31, 2016, we had \$779.8 million (undiscounted) principal amount of indebtedness, including \$375.0 million (undiscounted) of indebtedness under the 2018 Notes and \$375.0 million (undiscounted) principal amount of indebtedness under the 2020 Notes. Our indebtedness may:

- limit our ability to borrow additional funds for working capital, capital expenditures, acquisitions or other general business purposes;

- limit our ability to use our cash flow or obtain additional financing for future working capital, capital expenditures, acquisitions or other general business purposes;
- require us to use a substantial portion of our cash flow from operations to make debt service payments;
- limit our flexibility to plan for, or react to, changes in our business and industry;

45

---

- place us at a competitive disadvantage compared to our less leveraged competitors; and
- increase our vulnerability to the impact of adverse economic and industry conditions.

In addition, our ability to refinance our indebtedness will depend on the capital markets and our financial condition at such time.

Our indebtedness consists primarily of Notes, which, if not converted, will be required to be repaid in cash at maturity in 2018 and 2020. In addition, in the event the conditional conversion feature of the Notes is triggered, holders of Notes will be entitled to convert the Notes at any time during specified periods at their option. We intend to settle the principal amount of our conversion obligation in cash, which could adversely affect our liquidity. Even if holders do not elect to convert their Notes, we could be required under applicable accounting rules to reclassify all or a portion of the outstanding principal of the Notes as a current rather than long-term liability, which would result in a material reduction of our net working capital. Moreover, if we are unable to refinance the Notes, we must repay the Notes. While we could seek to obtain third-party financing to pay for any amounts due in cash upon such events, we cannot be sure that such third-party financing will be available on commercially reasonable terms, if at all. Furthermore, if we are required to share settle any conversions of Notes, due to lack of requisite liquidity or otherwise, we may cease to be eligible to account for the Notes using the treasury stock method, which may adversely impact our diluted earnings per share.

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 facility in the U.S. has been approved by the FDA, the European Commission (the EC), and health agencies in other countries for the manufacture of Aldurazyme, Naglazyme and Vimizim. Our manufacturing facility in Shanbally, Cork, Ireland has been approved by the FDA, the EC, and health agencies in other countries for the manufacture of Vimizim. 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 cGMP regulations.

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 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, Firdapse and Kyndrisa, if those manufacturers are unwilling or unable to fulfill their contractual obligations, we may be unable to meet demand for Kuvan and Firdapse or sell these products at all, we may lose potential revenue, and we may be forced to terminate a program. We have contracts for the production of final product for Kuvan, Firdapse and Kyndrisa. We also rely on third-parties for portions of the manufacture of Naglazyme, Aldurazyme and Vimizim. 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.

We manufacture Naglazyme, Aldurazyme and a portion of Vimizim in a manufacturing facility 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 manufacturers' ability to manufacture Kuvan or Firdapse.

Our Galli Drive facility located in Novato, California is currently our only manufacturing facility for Naglazyme and Aldurazyme and is one of two manufacturing facilities for 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.

We depend on single-source suppliers for critical raw materials and a limited number of manufacturing facilities to manufacture our finished products and product candidates. Numerous factors could cause interruptions in the supply or manufacture 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 suppliers and manufacturers as needed on a timely basis; and
- conditions affecting the cost and availability of raw materials.

If one of our suppliers or manufacturers fails or refuses to supply us with necessary raw materials or finished products or product candidates on a timely basis or at all, it would take a significant amount of time and expense to qualify a new supplier or

47

---

manufacturer. We may not be able to obtain active ingredients or finished products from new suppliers or manufacturers on acceptable terms and at reasonable prices, or at all.

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 in particular we must market worldwide to achieve significant market penetration of the product. In addition, because the number of potential patients in each disease population is 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.

Government authorities and other third-party payers are developing increasingly sophisticated methods of controlling healthcare costs, such as by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payers are requiring that drug companies provide them with predetermined discounts from list prices as a condition of coverage, are using restrictive formularies and preferred drug lists to leverage greater discounts in competitive classes, and are challenging the prices charged for medical products. Further, no uniform policy requirement for coverage and reimbursement for drug products exists among third-party payers in the U.S. Therefore, coverage and reimbursement for drug products can differ significantly from payer to payer. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payer separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

We cannot be sure that coverage and reimbursement will be available for any product that we commercialize or will continue to be available for any product that we have commercialized and, if reimbursement is available, what the level of reimbursement will be. Coverage and reimbursement may impact the demand for, or the price of, any product candidate for which we obtain marketing approval. If coverage and reimbursement are not available or reimbursement is available only to limited levels, we may not successfully commercialize any product candidate for which we obtain marketing approval or continue to market any product that has already been commercialized.

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 and Vimizim through special access or “named patient” programs, which do not require full product approval. 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. In particular, drug pricing by pharmaceutical companies has recently come under increased scrutiny and continues to be subject to intense political and public debate in the U.S. and abroad. 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. Specifically, there have been several recent U.S. Congressional inquiries and proposed bills designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. 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 or negative publicity related to our drug pricing or the pricing of pharmaceutical drugs generally could restrict the amount that we are able to charge for our current and future products or our sales volume, 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, among other things, 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 have affected us and increased 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, and created a new Patient-Centered Outcomes Research Institute to oversee clinical effectiveness research.

In addition, other legislative changes have been 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, following passage of the Bipartisan Budget Act of 2015, will remain in effect through 2025 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. Further, there have been judicial and Congressional challenges to certain aspects of the PPACA, and we expect there will be additional challenges and amendments to the PPACA in the future. 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 health care 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 laws and regulations, including anti-kickback laws, false claims laws, data privacy and security laws, and laws related to ensuring compliance. The federal Anti-Kickback Statute makes it illegal for any person or entity, 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. 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.

Federal and state false claims laws, including the civil False Claims Act, 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 (HIPAA), 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.

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations, including mandatory contractual terms, on certain types of individuals and entities, with respect to safeguarding the privacy, security and transmission of individually identifiable health information. Many state and foreign laws also govern the privacy and security of health information. They often differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Substantial new provisions affecting compliance have also been adopted, which may require us to modify our business practices with health care practitioners. The PPACA, through the Physician Payments Sunshine Act, requires drug manufacturers to collect and report to CMS 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.

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 Pharmaceutical Research and Manufacturers of America (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.

Due to 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, 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 Statute and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of these statutes or specific intent to violate them in order to commit a violation. 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 damages, fines, disgorgement, imprisonment, contractual damages, reputational harm, diminished profits and future earnings, curtailment of our operations, 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, Naglazyme and Vimizim, and all of the sales of Firdapse are generated from countries other than the U.S. We have operations in Canada and in several European, Middle Eastern, Asian, and Latin American 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:

- the increased complexity and costs inherent in managing international operations;
- diverse regulatory and compliance requirements, and changes in those 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;
- 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); and
- regulations relating to data security and the unauthorized use of, or access to, commercial and personal information.



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. 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 intellectual property, we may not be able to compete 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.

- Generic manufacturers may use litigation and regulatory means to obtain approval for generic versions of our products.
- 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 current and former 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

has an unpredictable outcome. 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.

Moreover, there is an increasing trend in the EU requiring public disclosure of development data, in particular clinical trial data. These data were traditionally regarded as confidential commercial information; however, under policies recently adopted in the EU, data submitted to the EMA in MAAs may be subject to public disclosure. Exactly how the new disclosure policy will be implemented is unclear; however, it could result in the EMA's public disclosure of certain of our clinical study reports, including pre-clinical data, and patient level data. The move toward public disclosure of development data could adversely affect our business in many ways, including, for example, resulting in the disclosure of our confidential methodologies for pre-clinical and clinical development of our products, preventing us from obtaining intellectual property right protection for innovations, requiring us to allocate significant resources to prevent other companies from violating our intellectual property rights, adding even more complexity to processing health data from clinical trials consistent with applicable data privacy regulations, and enabling competitors to use our data to gain approvals for their own products.

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 Kyndrisa and other antisense oligonucleotides, reveglucosidase alfa 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 BioMarin/Genzyme 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 BioMarin/Genzyme 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 BioMarin/Genzyme LLC at a specified buyout amount. If such option is not exercised, all rights to Aldurazyme will be sold and the BioMarin/Genzyme 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 BioMarin/Genzyme 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 BioMarin/Genzyme 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 BioMarin/Genzyme 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 BioMarin/Genzyme 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.

If we fail to develop new products and product candidates or compete successfully with respect to acquisitions, joint ventures, licenses or other collaboration opportunities, our ability to continue to expand our product pipeline and our growth and development would be impaired.

Our future growth and development depends in part on our ability to successfully develop new products from our research and development activities. The development of biopharmaceutical products is very expensive and time intensive and involves a great degree of risk. The outcomes of research and development programs, especially for innovative biopharmaceuticals, are inherently uncertain and may not result in the commercialization of any products.

Furthermore, our competitors compete with us to attract organizations for acquisitions, joint ventures, licensing arrangements or other collaborations. To date, several of our former and current product programs have been acquired through acquisitions, for example, reveglucosidase alfa and talazoparib (sold to Medivation in October 2015), and several of our former and current 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. Because 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 at any time after December 2011.

We own several patents that cover Kuvan, and we have listed those patents in conjunction with that product in the FDA's 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 may approve the ANDA if it is otherwise ready for approval. 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. In addition to our patent protection, we have received three-year Hatch-Waxman exclusivity for a New Patient Population for Kuvan that expires in October 2017, including pediatric exclusivity. Thus, depending on the proposed labeling of a generic product, generic versions of Kuvan 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.

We received a paragraph IV notice letter, dated October 3, 2014, from DRL, notifying us that DRL had filed an ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of our patents listed in the Orange Book. Additionally, we received a paragraph IV notice letter, dated January 22, 2015, from Par, notifying us that Par has filed an ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Together with Merck & Cie, on March 6, 2015 we filed lawsuits against both DRL and Par in the U.S. District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan tablets and seeking an injunction to prevent Par from introducing a generic version of Kuvan tablets that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of each ANDA in accordance with the Hatch-Waxman Act, which expires in July 2017. In response, DRL and Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid.

In September 2015, we entered into a settlement agreement with DRL that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral tablets. Under the terms of the settlement agreement, we have granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg tablets in the U.S. for the indications approved for Kuvan beginning at a confidential date in the future, but which is more than five years from the settlement date, or earlier under certain circumstances.

The settlement with DRL does not affect the case against Par, and the litigation against Par is still pending. The Court has set a claim construction hearing for May 5, 2016. The Court has not yet set a date for trial in the litigation against Par.

We also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying us that Par has filed a separate ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral powder prior to the expiration of our patents listed in the FDA's Orange Book. On February 22, 2016, we filed a lawsuit against Par in the U.S. District Court for the District of New Jersey alleging infringement of our patents relating to Kuvan powder and seeking an injunction to prevent Par from introducing a generic version of Kuvan powder that would infringe our patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of



Par's ANDA in accordance with the Hatch-Waxman Act, which expires in July 2018. In response, Par alleged, inter alia, that the asserted patents are not infringed and/or are invalid. The Court has set a scheduling conference for May 4, 2016.

The filing of DRL's and Par's purported ANDAs in respect to Kuvan could have an adverse impact on our stock price, and litigation to enforce our patents has, and is likely to continue to, cost a substantial amount and require significant management attention. If the patents covering Kuvan and its use are not upheld in litigation, or if 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. Moreover, generic competition from DRL following the settlement described above could 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 product candidates 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 MPS I, MPS VI, PKU or 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 our products and product candidates 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 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, 2016, 8% of our net product revenues were from Italy, Spain, Portugal, Greece and Russia. Approximately 14% of our total accounts receivable as of March 31, 2016, are related to these countries. 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 Rights to Kuvan and Pegvaliase (the PKU Franchise)

from Merck Serono

If we are unable to successfully integrate the expanded PKU franchise we acquired from Merck Serono into our existing business operations, our business could be adversely affected.

We will need to successfully integrate the PKU franchise rights we acquired from Merck Serono with our other business operations. Before the transaction with Merck Serono, we had exclusive rights to Kuvan in the U.S. and Canada and to pegvaliase in the U.S. and Japan. After the closing of the transaction with Merck Serono, we now have exclusive worldwide rights to Kuvan and pegvaliase, with the exception of Kuvan in Japan. Integrating the expanded PKU business with our existing business will be a complex and time-consuming process. There may be substantial difficulties, costs and delays involved in any integration of the expanded PKU business with that of our existing operations. These may include:

- distracting management from day-to-day operations;
- an inability to achieve synergies as planned;
- costs and delays in transitioning activities from Merck Serono, particularly with respect to the transfer of the Kuvan marketing authorizations;
- impact of unforeseen country level regulatory changes, delays and actions;
- difficulties in establishing distribution arrangements for Kuvan in all territories;
- reliance on Merck Serono to provide critical transition services for sales and distribution of Kuvan until marketing authorizations can be transferred in all countries;
- difficulties with respect to the timing and results of ongoing and future clinical trials of pegvaliase;
- an inability to manufacture both Kuvan and pegvaliase (pending regulatory approval) in the quantity and configuration required for each jurisdiction and intended use; and
- increased challenges in managing our business due to the global expansion of the PKU franchise, including, for example, generic competition to Kuvan.

Many of these risks may be accentuated because the acquired rights relate to activities 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 PKU franchise rights. The failure to integrate the expanded PKU business successfully would have a material adverse effect on our business, financial condition and results of operations.

The actual impact of the acquisition of the PKU franchise rights on our financial results may be worse than the assumptions we have used.

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

- the amount of intangible assets that will result from the acquisition;
- the impact of fair value adjustments to contingent acquisition consideration payable as a result of changes in estimated probability and timing of achieving the milestones;
- acquisition costs, including transaction and integration costs;
- the impact of impairment and other charges if the commercialization of Pegvaliase is unsuccessful; 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 us 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.

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

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

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;

58

---

- the amount of goodwill and intangibles that will result from the acquisition;
- the impact of fair value adjustments to contingent acquisition consideration payable as a result of changes in estimated probability and timing of achieving the milestones;
- the impact of additional impairment and other charges if the commercialization of Kyndrisa is unsuccessful;
- 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 of Prosensa.

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 of Prosensa 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 operate;
- complying with additional employment regulations in the new jurisdictions in which we 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.

If we are unable to commercialize Kyndrisa 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 our acquisition of Prosensa will depend heavily on the successful development and eventual commercialization of Kyndrisa, if approved.

In September 2013, Prosensa announced that the Phase 3 clinical trial of Kyndrisa 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 Kyndrisa, including retrospective and subgroup analyses, provide strong support for concluding that Kyndrisa showed clinically meaningful improvements over placebo in these trials, we cannot be sure that Prosensa's data will be sufficient to obtain regulatory approval in any jurisdiction.

In January 2016 the FDA issued a complete response letter to our NDA for Kyndrisa for the treatment of DMD amenable to exon 51 skipping. The FDA issues complete response letters to indicate that the review cycle for an application is complete and that the application is not ready for approval in its present form. The FDA concluded that the standard of substantial evidence of effectiveness for Kyndrisa had not been met. An MAA for Kyndrisa for the treatment of DMD amenable to exon 51 skipping remains under review in the EU. We anticipate that the CHMP of the EMA will provide an opinion for our MAA for Kyndrisa in the second quarter of 2016. If the CHMP opinion is positive, the MAA will be referred to the EC. The EC is expected to render a final decision for Kyndrisa in the second half of 2016. If the MAA is not approved by the EC, we will not receive marketing authorization for Kyndrisa in the EU and will be unable to sell Kyndrisa in the EU or roll out our international registration strategy for Kyndrisa using the EU approval as the basis of multiple international applications. If our current applications for marketing approval are denied, we may need to conduct additional clinical trials at significant delay and cost or abandon development of Kyndrisa altogether. If we are ultimately unable to obtain regulatory approval to commercially market and sell Kyndrisa, we may need to write-off all or a significant portion of the costs associated with our acquisition of Prosensa in addition to the impairment charge we recorded in 2015.

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

- we may not achieve market acceptance of Kyndrisa by physicians, patients and third-party payers;
- Kyndrisa may not have an acceptable safety profile following approval;
- we may not be able to manufacture Kyndrisa 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 Kyndrisa 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 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 Kyndrisa 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 Kyndrisa, Prosensa performed retrospective and subgroup analyses of the Phase 3 clinical trial and prior Phase 2 clinical trials of Kyndrisa that we believe provide strong support for concluding that Kyndrisa 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 additional clinical trials of Kyndrisa or may need to abandon development of Kyndrisa altogether if our current applications for marketing approval are denied.

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 Kyndrisa and our other product candidates acquired from Prosensa. If we are required to conduct additional clinical trials of Kyndrisa, 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.

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

Our valuation and stock price have 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;
- manufacturing, supply or distribution of Vimizim, Naglazyme, Kuvan, Aldurazyme and Firdapse;
- progress of our integration of the PKU franchise rights acquired from Merck Serono;
- 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 lawsuits against Par to protect our patents relating to Kuvan and generic competition to Kuvan relating to our settlement with DRL;
- 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;
- changes in company assessments or financial estimates by securities analysts; and
- sales of our shares of stock by us, our significant shareholders, or members of our management or Board of Directors.

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.

Conversion of the Notes will dilute the ownership interest of existing stockholders, including holders who had previously converted their Notes, or may otherwise depress the price of our common stock.

The conversion of some or all of the Notes will dilute the ownership interests of existing stockholders to the extent we deliver shares upon conversion of any of the Notes. The Notes may become in the future convertible at the option of their holders prior to their scheduled terms under certain circumstances. Any sales in the public market of the common stock issuable upon such conversion could adversely affect prevailing market prices of our common stock. In addition, the existence of the Notes may encourage short selling by market participants because the conversion of the Notes could be used to satisfy short positions, or anticipated conversion of the Notes into shares of our common stock could depress the price of our common stock.

The capped call transactions may affect the value of the Notes and our common stock.

In connection with the issuance of the 2018 Notes and 2020 Notes, we entered into capped call transactions with respect to 50% of the principal amount of the 2018 Notes and 50% of the principal amount of the 2020 Notes with certain hedge counterparties. The capped call transactions will cover, subject to customary anti-dilution adjustments, the aggregate number of shares of common stock underlying 50% of the principal amount of the relevant Notes and are expected generally to reduce potential dilution to the common stock upon conversion of the relevant Notes in excess of the principal amount of such converted Notes. In connection with establishing their initial hedges of the capped call transactions, the hedge counterparties (or their affiliates) entered into various derivative transactions with respect to the common stock concurrently with, and/or purchased the common stock shortly after, the pricing of the relevant notes. The hedge counterparties (or their affiliates) are likely to modify their hedge positions by entering into or unwinding various derivative transactions with respect to the common stock and/or by purchasing or selling the common stock or other securities of ours in secondary market transactions prior to the maturity of the relevant Notes (and are likely to do so during the settlement averaging period under the relevant capped call transactions, which precedes the maturity date of the relevant Notes, and on or around any earlier conversion date related to a conversion of the relevant Notes).

The effect, if any, of any of these transactions and activities on the market price of our common stock or the Notes will depend in part on market conditions and cannot be ascertained at this time, but any of these activities could adversely affect the value of our common stock, which could affect the value of the Notes and the value of our common stock, if any, that Note holders receive upon any conversion of the Notes.

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.

The fundamental change repurchase feature of the Notes may delay or prevent an otherwise beneficial attempt to take over our company.

The terms of the Notes require us to repurchase the Notes in the event of a fundamental change. A takeover of our company would trigger options by the respective holders of the applicable Notes to require us to repurchase such Notes. This may have the effect of delaying or preventing a takeover of our company that would otherwise be beneficial to our stockholders or investors in the Notes.

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures

None.

Item 5. Other Information.

None.

Item 6. Exhibits.

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, 2016 and December 31, 2015, (ii) Condensed Consolidated Statements of Comprehensive Loss for the three months ended March 31, 2016 and 2015,
- (iii) Condensed Consolidated Statements of Cash Flows for the three months ended March 31, 2016 and 2015, 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 2, 2016

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, 2016 and December 31, 2015, (ii) Condensed Consolidated Statements of Comprehensive Loss for the three months ended March 31, 2016 and 2015, (iii) Condensed Consolidated Statements of Cash Flows for the three months ended March 31, 2016 and 2015, and (iv) Notes to Condensed Consolidated Financial Statements.