BIOMARIN PHARMACEUTICAL INC
Form 10-Q
August 02, 2017

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-Q

(Mark One)

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

Or

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

Commission File Number: 000-26727

BioMarin Pharmaceutical Inc.

(Exact name of registrant as specified in its charter)

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

| | |
|---|---|
| 770 Lindaro Street, San Rafael, California | 94901 |
| (Address of principal executive offices) | (Zip Code) |

(415) 506-6700

(Registrant's telephone number including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes 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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer Accelerated filer

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

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

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

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: 175,268,321 shares of common stock, par value $0.001, outstanding as of July 20, 2017.

BIOMARIN PHARMACEUTICAL INC.

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Unless the context suggests otherwise, references in this Quarterly Report on Form 10-Q to "BioMarin," the "Company," "we," "us," and "our" refer to BioMarin Pharmaceutical Inc. and, where appropriate, its wholly owned subsidiaries.

BioMarin®, Vimizim®, Naglazyme®, Kuvan® and Firdapse® are our registered trademarks. Brineura™ and Kyndrisa™ are our 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.

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," "m "will," "projects," "continues," "estimates," "potential," "opportunity" or the negative versions of these terms and other similar expressions. 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, 2016, which was filed with the Securities and Exchange Commission (the SEC) on February 27, 2017. 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 the Company's management based on information currently available to management and should be considered in connection with any written or oral forward-looking statements that the Company may issue in the future as well as other cautionary statements the Company has made and may make. Except as required by law, the Company does 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 discussion of the Company's financial condition and results of operations should be read in conjunction with the Company's Condensed Consolidated Financial Statements and the related Notes thereto included in this Quarterly Report on Form 10-Q.

PART I. FINANCIAL INFORMATION

Item 1. Financial Statements

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

June 30, 2017 and December 31, 2016

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

| | June 30, 2017 | December 31, 2016[1] |
|---|---|---|
| ASSETS | (unaudited) | |
| Current assets: | | |
| Cash and cash equivalents | $354,864 | $408,330 |
| Short-term investments | 372,912 | 381,347 |
| Accounts receivable, net | 238,338 | 215,280 |
| Inventory | 429,831 | 355,126 |
| Other current assets | 62,875 | 61,708 |
| Total current assets | 1,458,820 | 1,421,791 |
| Noncurrent assets: | | |
| Long-term investments | 482,036 | 572,711 |
| Property, plant and equipment, net | 851,097 | 798,768 |
| Intangible assets, net | 538,565 | 553,780 |
| Goodwill | 197,039 | 197,039 |
| Deferred tax assets | 470,961 | 446,786 |
| Other assets | 21,447 | 32,815 |
| Total assets | $4,019,965 | $4,023,690 |
| LIABILITIES AND STOCKHOLDERS' EQUITY | | |
| Current liabilities: | | |
| Accounts payable and accrued liabilities | $319,210 | $370,505 |
| Short-term convertible debt, net | — | 22,478 |
| Short-term contingent acquisition consideration payable | 55,093 | 46,327 |
| Total current liabilities | 374,303 | 439,310 |
| Noncurrent liabilities: | | |
| Long-term convertible debt, net | 676,205 | 660,761 |
| Long-term contingent acquisition consideration payable | 122,899 | 115,310 |
| Other long-term liabilities | 50,979 | 42,034 |
| Total liabilities | 1,224,386 | 1,257,415 |
| Stockholders' equity: | | |
| Common stock, $0.001 par value: 500,000,000 shares authorized; 175,248,847 and 172,647,588 shares issued and outstanding as of June 30, 2017 and December 31, | 175 | 173 |

| | | |
|---|---|---|
| 2016, respectively. | | |
| Additional paid-in capital | 4,397,980 | 4,288,113 |
| Company common stock held by Nonqualified Deferred Compensation Plan (NQDC) | (14,289) | (14,321) |
| Accumulated other comprehensive income (loss) | (14,658) | 12,816 |
| Accumulated deficit | (1,573,629) | (1,520,506) |
| Total stockholders' equity | 2,795,579 | 2,766,275 |
| Total liabilities and stockholders' equity | $4,019,965 | $4,023,690 |

(1) December 31, 2016 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, 2016, filed with the Securities and Exchange Commission (the SEC) on February 27, 2017.

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

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

Three and Six Months Ended June 30, 2017 and 2016

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

(Unaudited)

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2017 | 2016 | 2017 | 2016 |
| REVENUES: | | | | |
| Net product revenues | $315,926 | $298,576 | $618,116 | $533,933 |
| Royalty and other revenues | 1,522 | 1,555 | 3,077 | 2,934 |
| Total revenues | 317,448 | 300,131 | 621,193 | 536,867 |
| OPERATING EXPENSES: | | | | |
| Cost of sales | 56,305 | 51,617 | 106,311 | 94,735 |
| Research and development | 143,039 | 167,039 | 288,042 | 325,832 |
| Selling, general and administrative | 143,505 | 109,577 | 263,524 | 214,877 |
| Intangible asset amortization and contingent consideration | 13,411 | (54,414) | 22,336 | (43,972) |
| Impairment of intangible assets | — | 599,118 | — | 599,118 |
| Total operating expenses | 356,260 | 872,937 | 680,213 | 1,190,590 |
| LOSS FROM OPERATIONS | (38,812) | (572,806) | (59,020) | (653,723) |
| Equity in the loss of BioMarin/Genzyme LLC | (220) | (135) | (743) | (270) |
| Interest income | 2,983 | 1,357 | 6,055 | 2,928 |
| Interest expense | (10,040) | (9,944) | (20,159) | (19,787) |
| Other income (expense) | 543 | (1,417) | 4,015 | (1,219) |
| LOSS BEFORE INCOME TAXES | (45,546) | (582,945) | (69,852) | (672,071) |
| Benefit from income taxes | (8,713) | (163,931) | (16,729) | (170,006) |
| NET LOSS | $(36,833) | $(419,014) | $(53,123) | $(502,065) |
| NET LOSS PER SHARE, BASIC AND DILUTED | $(0.21) | $(2.58) | $(0.31) | $(3.10) |
| Weighted average common shares outstanding, basic and diluted | 174,374 | 162,587 | 173,547 | 162,067 |
| COMPREHENSIVE LOSS | $(56,511) | $(414,533) | $(80,597) | $(518,570) |

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

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENT OF STOCKHOLDERS' EQUITY

Six Months Ended June 30, 2017

(In thousands of U.S. dollars)

(Unaudited)

| | Common stock | | Additional | Company Common Stock | Accumulated Other | | Total |
|---|---|---|---|---|---|---|---|
| | Shares | Amount | Paid-in Capital | Held by NQDC | Comprehensive Income (Loss) | Accumulated Deficit | Stockholders' Equity |
| Balance at December 31, 2016 | 172,648 | $ 173 | $4,288,113 | $(14,321) | $ 12,816 | $(1,520,506) | $2,766,275 |
| Net loss | — | — | — | — | — | (53,123) | (53,123) |
| Other comprehensive loss | — | — | — | — | (27,474) | — | (27,474) |
| Issuances under equity incentive plans, net of tax | 1,402 | 1 | 7,330 | — | — | — | 7,331 |
| Issuances of common stock under the Employee Stock Purchase Plan (the ESPP) | 95 | — | 6,704 | — | — | — | 6,704 |
| Conversion of convertible notes, net | 1,104 | 1 | 22,476 | — | — | — | 22,477 |
| Common stock held by NQDC | — | — | — | 32 | — | — | 32 |
| Stock-based compensation | — | — | 73,357 | — | — | — | 73,357 |
| Balance at June 30, 2017 | 175,249 | $ 175 | $4,397,980 | $(14,289) | $ (14,658) | $(1,573,629) | $2,795,579 |

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

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

Six Months Ended June 30, 2017 and 2016

(In thousands of U.S. dollars)

(Unaudited)

| | 2017 | 2016 |
|---|---|---|
| CASH FLOWS FROM OPERATING ACTIVITIES: | | |
| Net loss | $(53,123) | $(502,065) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Depreciation and amortization | 38,497 | 47,383 |
| Non-cash interest expense | 15,601 | 14,762 |
| Accretion of discount on investments | 1,327 | 536 |
| Stock-based compensation | 70,775 | 64,075 |
| Gain on the sale of equity investments | (3,252) | 2,027 |
| Impairment of intangible assets | — | 599,118 |
| Deferred income taxes | (22,770) | (191,131) |
| Unrealized foreign exchange gain on forward contracts | (4,870) | (7,882) |
| Non-cash changes in the fair value of contingent acquisition consideration payable | 7,195 | (59,066) |
| Other | 3,806 | 705 |
| Changes in operating assets and liabilities: | | |
| Accounts receivable, net | (16,834) | (50,250) |
| Inventory | (60,369) | (41,600) |
| Other current assets | (3,710) | 3,186 |
| Other assets | (1,109) | (1,439) |
| Accounts payable and accrued liabilities | (36,286) | (87,560) |
| Other long-term liabilities | 3,459 | (8,058) |
| Net cash used in operating activities | (61,663) | (217,259) |
| CASH FLOWS FROM INVESTING ACTIVITIES: | | |
| Purchases of property, plant and equipment | (116,847) | (70,710) |
| Maturities and sales of investments | 234,617 | 283,780 |
| Purchase of available-for-sale securities | (130,986) | (58,914) |
| Business acquisitions, net of cash acquired | — | (1,467) |
| Other | (1,560) | (150) |
| Net cash (used in) provided by investing activities | (14,776) | 152,539 |
| CASH FLOWS FROM FINANCING ACTIVITIES: | | |
| Proceeds from exercises of stock options and the ESPP | 40,659 | 23,014 |
| Taxes paid related to net share settlement of equity awards | (26,624) | (52,824) |
| Payment of contingent acquisition consideration payable | (1,894) | — |
| Other | (28) | — |
| Net cash provided by (used in) financing activities | 12,113 | (29,810) |
| Effect of exchange rate changes on cash | 10,860 | 3,459 |
| NET DECREASE IN CASH AND CASH EQUIVALENTS | (53,466) | (91,071) |
| Cash and cash equivalents: | | |
| Beginning of period | $408,330 | $397,040 |

| | | |
|---|---|---|
| End of period | $354,864 | $305,969 |
| SUPPLEMENTAL CASH FLOW DISCLOSURES: | | |
| Cash paid for interest, net of interest capitalized into fixed assets | 4,519 | 4,521 |
| Cash paid for income taxes | 16,341 | 93,969 |
| Stock-based compensation capitalized into inventory | 7,600 | 5,751 |
| Depreciation capitalized into inventory | 11,752 | 8,993 |
| SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON CASH INVESTING AND FINANCING ACTIVITIES: | | |
| Decrease in accounts payable and accrued liabilities related to fixed assets | (29,300) | (17,130) |
| Conversion of convertible debt | 22,477 | 6,941 |
| Accrual for inventory purchases related to the acquisition of the Merck PKU Business | — | 1,322 |

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

BIOMARIN PHARMACEUTICAL INC.

NOTES TO UNAUDITED 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) is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare diseases and medical conditions. The Company 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 therapy portfolio consists of six approved products and multiple clinical and pre-clinical product candidates.

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 through proceeds from debt or equity offerings, commercial borrowing, or through 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 commercial products; the potential need for additional financings; the Company's ability to successfully commercialize its approved products; 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. Please see "Risk Factors" included in Part II, Item 1A of this Quarterly Report on Form 10-Q for a more detailed discussion of these risks.

(2) BASIS OF PRESENTATION

The accompanying Condensed Consolidated Financial Statements have been prepared pursuant to United States generally accepted accounting principles (U.S. GAAP) and the rules and regulations of the SEC for Quarterly Reports on Form 10-Q and do not include all of the information and note disclosures required by U.S. 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, 2016 included in the Company's Annual Report on Form 10-K.

U.S. GAAP 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 and six months ended June 30, 2017 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2017 or any other period.

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, except for the transaction disclosed in Note 20.

(3) SIGNIFICANT ACCOUNTING POLICIES

There have been no material changes to the Company's significant accounting policies during the six months ended June 30, 2017, 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, 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)

(4) RECENT ACCOUNTING PRONOUNCEMENTS

Except as described below, there have been no new accounting pronouncements or changes to accounting pronouncements during the six months ended June 30, 2017, 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, 2016, that the Company believes are of significance or potential significance to the Company.

In May 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (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 No. 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. In 2016 and 2017, the FASB issued several ASUs to help provide interpretive clarifications on the new guidance for ASC Topic 606. The guidance may be applied retrospectively to each prior period presented (full retrospective method), or with the cumulative effect recognized as of the date of initial adoption (modified retrospective method).

As of June 30, 2017, the Company has not elected early adoption and has not concluded on an adoption method. The Company has formed a task force that is in the process of analyzing the Company's customer contracts and the potential impacts the standard may have on previously reported revenues and future revenues. After completing the analysis of the accounting for the Company's customer contracts under the new revenue standard, management will assess the required changes to the Company's accounting policies, systems and internal control over financial reporting. Based on management's preliminary analysis of the Company's material contracts with customers, management does not anticipate that ASU 2014-09 will have a material impact on the timing of revenue recognition for the products that are marketed by the Company (all products except for Aldurazyme). Management is still assessing the application of ASU 2014-09 to Aldurazyme revenues earned from Genzyme Corporation (Genzyme), which are currently recognized in two components: upon delivery to Genzyme and upon sale of the product by Genzyme to third parties. ASU 2014-09 may have an impact on the timing of Aldurazyme revenue recognition, however management is in the early stages of the analysis and has not yet concluded on the impact the new revenue standard will have on the timing of revenue recognition for Aldurazyme.

In January 2016, the FASB issued ASU No. 2016-01 (ASU 2016-01), Recognition and Measurement of Financial Assets and Financial Liabilities. ASU 2016-01 changes accounting for equity investments, financial liabilities under the fair value option and the presentation and disclosure requirements for financial instruments. In addition, the update clarifies guidance related to the valuation allowance assessment when recognizing deferred tax assets resulting from unrealized losses on available-for-sale debt securities. The guidance will become effective for the Company's fiscal year beginning January 1, 2018 and must be adopted using a modified retrospective approach, with certain exceptions. Early adoption is permitted for certain provisions. The Company is currently evaluating the impact that the standard will have on its Consolidated Financial Statements. As of June 30, 2017, the Company has not elected to early adopt the amendments of ASU 2016-01.

In February 2016, the FASB issued ASU No. 2016-02, Leases (ASU 2016-02). The amended guidance requires balance sheet recognition of lease right-of-use (ROU) assets and liabilities by lessees for leases classified as operating leases, with an option to not recognize lease ROU 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 adoption is permitted, but, as of June 30, 2017, the Company has not made the election to do so. ASU 2016-02 will be effective for the Company’s fiscal year beginning January 1, 2019. The amendments require a modified retrospective approach with optional practical expedients.

As of June 30, 2017, the Company has formed a task force that is in the process of analyzing the Company’s lease contracts and the potential impacts the standard may have on its Consolidated Financial Statements and related disclosures. After completing the analysis of the accounting for the Company’s lease contracts under the amendments, management will assess the required changes to the Company’s accounting policies, systems and internal control over financial reporting. Based on management’s preliminary analysis, the Company anticipates the amendments will have a material impact on the Company’s Consolidated Balance Sheets due to the requirement to recognize lease ROU assets and corresponding liabilities related to leases on the Company’s Consolidated Balance Sheets, but they are not anticipated to have a material impact on the Company’s other Consolidated Financial Statements.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

In May 2017 the FASB issued ASU No. 2017-09, Compensation - Stock Compensation (Topic 718): Scope of Modification Accounting (ASU 2017-09). The amendment provides clarification about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Topic 718. ASU 2017-09 is effective for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years. Early adoption is permitted and, as of June 30, 2017, the Company has made the election to do so as its policies have already been in compliance with the amendments. The adoption of this standard is not expected to have a material impact on the Company's Consolidated Financial Statements.

(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 the Company had granted to Merck Serono under the License Agreement. Also on October 1, 2015, the Company and Merck Serono entered into a Termination Agreement (the Pegvaliase Agreement) to terminate the license to pegvaliase the Company had granted to Merck Serono under the License Agreement. 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 the Merck PKU Business, the Company recognized transaction costs of $0.6 million, of which $0.3 million was recognized in each of the years ended December 31, 2016 and 2015.

Pursuant to the A&R Kuvan Agreement, the Company paid Merck Serono $374.5 million, in cash, the majority of which was paid in January 2016, 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. As of December 31, 2016, the inventory acquired from Merck Serono had been sold through to customers. The Company and Merck Serono have no further rights or obligations under the License Agreement with respect to Kuvan or pegvaliase.

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) and/or for primary BH4 deficiency in certain countries. At the time of the acquisition, pegvaliase was in pivotal studies as a potential therapeutic option for adult patients with PKU. In March 2016, the Company announced that its pivotal Phase 3 PRISM-2 study of pegvaliase met the primary endpoint of change in blood Phe compared with placebo ($p<0.0001$); and the Company submitted a marketing application in the U.S. in June 2017 and announced its plans to submit an application for registration in the European Union (EU). Kuvan has Orphan Drug exclusivity in the EU until 2020, and pegvaliase has Orphan Drug designation in the U.S. and the 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 as of June 30, 2017. See Note 13 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.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

The following table presents the final allocation of the purchase consideration for the Merck PKU Business acquisition, including the contingent acquisition consideration payable based on the acquisition date fair value. The allocation of the purchase price below reflects an inventory adjustment in the second quarter of 2016.

| | |
|---|---|
| Cash payments | $374,545 |
| Estimated fair value of contingent acquisition consideration payable | 138,974 |
| Total consideration | $513,519 |

| | |
|---|---|
| Kuvan intangible assets | $172,961 |
| Pegvaliase IPR&D | 326,359 |
| Inventory | 14,199 |
| Total identifiable assets acquired | $513,519 |

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.

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.

(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 potential shares of common stock that were excluded from the computation of basic and diluted earnings per common share as they were anti-dilutive using the if-converted or treasury stock method (in thousands):

| | Three and Six Months Ended June 30, | |
|---|---|---|
| | 2017 | 2016 |
| Options to purchase common stock | 8,440 | 10,445 |
| Common stock issuable under the 2017 Notes | — | 1,203 |
| Common stock issuable under the 2018 and 2020 Notes | 7,966 | 7,966 |
| Unvested restricted stock units | 3,041 | 2,829 |
| Common stock potentially issuable for ESPP purchases | 387 | 356 |
| Common stock held by the NQDC | 224 | 253 |
| Total number of potentially issuable shares | 20,058 | 23,052 |

The effect of the Company's 0.75% senior subordinated convertible notes due in 2018 (the 2018 Notes) and the Company's 1.50% senior subordinated convertible notes due in 2020 (the 2020 Notes, and together with the 2018 Notes, the Notes) were excluded from the diluted net loss per common share because they were antidilutive using the if-converted method. The Company's closing stock price on June 30, 2017 and 2016 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) AVAILABLE-FOR-SALE SECURITIES

All investments were classified as available-for-sale at June 30, 2017 and December 31, 2016. 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 June 30, 2017 and December 31, 2016 are summarized in the tables below:

| | Amortized Cost | Gross Unrealized Holding Gains | Gross Unrealized Holding Losses | Aggregate Fair Value at June 30, 2017 |
|---|---|---|---|---|
| Corporate debt securities | $ 615,435 | $ 426 | $ (1,411) | $ 614,450 |
| Commercial paper | 8,976 | — | — | 8,976 |
| U.S. government agency securities | 233,898 | 4 | (840) | 233,062 |
| Greek government-issued bonds | 48 | 122 | (1) | 169 |
| Total | $ 858,357 | $ 552 | $ (2,252) | $ 856,657 |

| | Amortized Cost | Gross Unrealized Holding Gains | Gross Unrealized Holding Losses | Aggregate Fair Value at December 31, 2016 |
|---|---|---|---|---|
| Certificates of deposit | $ 2,800 | $ — | $ — | $ 2,800 |
| Corporate debt securities | 641,670 | 329 | (2,282) | 639,717 |
| Commercial paper | 16,075 | — | — | 16,075 |
| U.S. government agency securities | 310,635 | 37 | (747) | 309,925 |
| Greek government-issued bonds | 48 | 86 | — | 134 |
| Total | $ 971,228 | $ 452 | $ (3,029) | $ 968,651 |

As of December 31, 2016, the Company had one investment in marketable equity securities, measured using quoted prices in its active market, which was considered a strategic investment. In the first quarter of 2017, the strategic investment was sold for a realized gain of $3.3 million. As of December 31, 2016, the fair value of the Company's marketable equity securities was $4.1 million, which included an unrealized gain of $2.3 million, and was recorded in Other Assets in the Company's Condensed Consolidated Balance Sheet.

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

| | June 30, 2017 | December 31, 2016 |
|---|---|---|
| Maturing in one year or less | $374,621 | $ 395,940 |
| Maturing after one year through five years | 482,036 | 572,711 |
| Total | $856,657 | $ 968,651 |

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 June 30, 2017, some of the Company's investments were in an unrealized loss position, which the Company considers temporary in nature. 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:

| | June 30, 2017 | December 31, 2016 |
|---|---|---|
| Intangible assets: | | |
| Finite-lived intangible assets | $303,297 | $ 305,122 |
| Indefinite-lived intangible assets | 332,199 | 332,199 |
| Gross intangible assets: | 635,496 | 637,321 |
| Less: Accumulated amortization | (96,931 ) | (83,541 ) |
| Net carrying value | $538,565 | $ 553,780 |

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.

During the second quarter of 2016, the Company recorded impairment charges of $574.1 million based on the status of development efforts. These impairments reduced the remaining book value of certain IPR&D assets to zero due to the termination of the Kyndrisa and other exon programs. During the three and six months ended June 30, 2016, the Company also recognized an impairment charge of $25.0 million related to the reveglucosidase alfa IPR&D assets due to the decision to terminate that development program. When a triggering event occurs, management evaluates both IPR&D assets and goodwill for possible impairments. Although management concluded these IPR&D assets were impaired as of June 30, 2016, management determined that goodwill was not impaired as of June 30, 2016. Because the Company’s single reporting unit is the consolidated entity, management compares the total carrying value of the single reporting unit, including goodwill, to the fair value of the reporting unit, as evidenced by the Company’s market capitalization. As of June 30, 2016, the Company’s capitalization exceeded the carrying value of its single reporting unit supporting management’s conclusion that goodwill was not impaired.

See Note 7 to the Consolidated Financial Statements included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2016 for additional information related to the Company’s intangible assets.

(9) PROPERTY, PLANT AND EQUIPMENT

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

| | June 30, 2017 | December 31, 2016 |
|---|---|---|
| Building and improvements | $555,868 | $ 510,805 |
| Manufacturing and laboratory equipment | 261,616 | 242,899 |
| Computer hardware and software | 137,041 | 129,506 |
| Leasehold improvements | 43,614 | 44,184 |
| Furniture and equipment | 28,370 | 27,229 |
| Land improvements | 4,881 | 4,881 |
| Land | 56,050 | 55,412 |
| Construction-in-progress | 135,159 | 126,446 |
| | 1,222,599 | 1,141,362 |
| Less: Accumulated depreciation | (371,502) | (342,594) |
| Total property, plant and equipment, net | $851,097 | $ 798,768 |

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 construction-in-process balance primarily includes costs related to the Company's significant in-process projects at its facilities in Marin County, California, and in Shanbally, Cork, Ireland.

Depreciation expense for the three and six months ended June 30, 2017 was $17.9 million and $35.4 million, respectively, of which $6.2 million and $11.8 million, respectively, was capitalized into inventory. Depreciation expense for the three and six months ended June 30, 2016 was $17.2 million and $32.9 million, respectively, of which $4.8 million and $9.0 million, respectively, was capitalized into inventory. Capitalized interest related to the Company's property, plant and equipment purchases for each of the three and six months ended June 30, 2017 and 2016 was insignificant.

(10) SUPPLEMENTAL BALANCE SHEET INFORMATION

Inventory consisted of the following:

| | June 30, 2017 | December 31, 2016 |
|---|---|---|
| Raw materials | $46,602 | $ 51,250 |
| Work-in-process | 253,658 | 167,788 |
| Finished goods | 129,571 | 136,088 |
| Total inventory | $429,831 | $ 355,126 |

In the third quarter of 2016, process qualification production activities commenced in the Company's Shanbally facility related to the Brineura manufacturing process. As of June 30, 2017, the value of the Shanbally qualification campaign was $22.7 million, 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 Brineura, should that occur, the value of the inventory would be expensed at that time.

Accounts Payable and Accrued Liabilities consisted of the following:

| | June 30, 2017 | December 31, 2016 |
|---|---|---|
| Accounts payable and accrued operating expenses | $158,229 | $ 191,353 |
| Accrued compensation expense | 80,604 | 109,038 |
| Accrued rebates payable | 37,973 | 34,737 |
| Accrued royalties payable | 15,541 | 15,151 |
| Value added taxes payable | 8,609 | 7,848 |
| Other | 18,254 | 12,378 |
| Total accounts payable and accrued liabilities | $319,210 | $ 370,505 |

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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) DEBT

Convertible Notes

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

| | June 30, 2017 | December 31, 2016 |
|---|---|---|
| Convertible Notes due in 2017 | $— | $ 22,503 |
| Unamortized deferred offering costs | — | (25) |
| Convertible Notes due in 2017, net | — | 22,478 |
| | | |
| Convertible Notes due in 2018 | 374,980 | 374,980 |
| Unamortized discount | (20,122) | (27,566) |
| Unamortized deferred offering costs | (2,515) | (3,484) |
| Convertible Notes due in 2018, net | 352,343 | 343,930 |
| | | |
| Convertible Notes due in 2020 | 374,993 | 374,993 |
| Unamortized discount | (46,854) | (53,239) |
| Unamortized deferred offering costs | (4,277) | (4,923) |
| Convertible Notes due in 2020, net | 323,862 | 316,831 |
| | | |
| Total convertible debt, net | $676,205 | $ 683,239 |
| | | |
| Fair value of fixed rate convertible debt | | |
| Convertible Notes due in 2017 [1] | $— | $ 90,977 |
| Convertible Notes due in 2018 [1] | 416,337 | 423,202 |
| Convertible Notes due in 2020 [1] | 446,725 | 442,754 |
| Total | $863,062 | $ 956,933 |

(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 June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2017 | 2016 | 2017 | 2016 |
| Coupon interest | $2,194 | $2,520 | $4,558 | $5,026 |
| Amortization of issuance costs | 886 | 825 | 1,772 | 1,649 |
| Accretion of debt discount | 6,960 | 6,599 | 13,829 | 13,112 |

| | | | | |
|---|---|---|---|---|
| Total interest expense on convertible debt | $10,040 | $9,944 | $20,159 | $19,787 |

In April 2017, the remaining $22.5 million of the Company's senior subordinated notes due in 2017 (the 2017 Notes) matured and were converted into 1,103,704 shares of the Company's common stock. During three and six months ended June 30, 2016, certain existing holders of the Company's senior subordinated notes due in 2017 elected to convert $6.9 million in aggregate principal amount of the 2017 Notes into 340,967 shares of the Company's common stock.

See Note 13 to the Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2016 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)

Revolving Credit Facility

In November 2016, the Company entered into a credit agreement (Credit Agreement) with Bank of America, N.A., as the administrative agent, swing line lender and letter of credit issuer. The Credit Agreement provides for up to $100.0 million in revolving loans (Revolving Credit Facility), a $10.0 million letter of credit subfacility and a $15.0 million swing line loan subfacility. The maturity date of the Revolving Credit Facility will occur on November 29, 2018. Interest on any outstanding balance of the Revolving Credit Facility is payable quarterly and draws may be voluntary prepaid at any time without penalty. In connection with entering into the Credit Agreement, $0.6 million in financing costs were incurred and will be amortized as Interest Expense over the term of the Credit Agreement. As of June 30, 2017 and December 31, 2016, there were no outstanding amounts due under the Revolving Credit Facility.

In connection with the Revolving Credit Facility, the Company and certain of its subsidiaries are required to comply with covenants, including, among other things, restrictions on the Company's and such subsidiaries' ability to incur additional indebtedness, dispose of its assets, incur liens, make investments, and pay dividends or other distributions, in each case subject to specified exceptions. The Credit Agreement also contains customary indemnification obligations and customary events of default. If the Company's Global Liquidity, which is defined as the sum of the market value of unrestricted cash, marketable securities and other assets to the extent constituting "cash and cash equivalents," "short-term investments" or "long-term investments" as reflected in the Company's Condensed Consolidated Balance Sheet, in each case, held by the Company or certain of the Company's subsidiaries at such time, regardless of where such assets are domiciled, falls below $225.0 million at the end of any month or at the time of any borrowing or issuance of a letter of credit under the Revolving Credit Facility, then the Company's obligations under the Credit Agreement will also be secured by the assets held by the Company in the custody account, which was established in the first quarter of 2017. As of June 30, 2017, the Company and certain of its subsidiaries that serve as guarantors were in compliance with all covenants.

(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 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.

The Company enters into forward foreign currency exchange contracts 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 operating expenses denominated in Euros and revenues denominated in currencies other than the U.S. dollar related to changes in foreign currency exchange rates.

BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

The following table summarizes the Company's designated forward foreign currency exchange contracts outstanding as of June 30, 2017 (notional amounts in millions):

| Foreign Exchange Contracts | Number of Contracts | Aggregate Notional Amount in Foreign Currency | Maturity |
|---|---|---|---|
| Euros – Purchase | 72 | 114.3 | Jul. 2017 - Jun. 2020 |
| Euros – Sell | 300 | 379.6 | Jul. 2017 - Jun. 2020 |
| Canadian Dollars – Sell | 12 | 11.9 | Jul. 2017 - Dec. 2017 |
| Colombian Pesos – Sell | 6 | 31,152.0 | Jul. 2017 - Dec. 2017 |
| Brazilian Reais – Sell | 2 | 33.0 | Oct. 2017 |
| Total | 392 | | |

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 June 2020. Over the next twelve months, the Company expects to reclassify unrealized losses of $9.2 million from accumulated other comprehensive income (loss) to earnings as the forecasted revenue and operating expense transactions occur.

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.

The following table summarizes the Company's non-designated forward foreign currency exchange contracts outstanding as of June 30, 2017 (notional amounts in millions):

| Foreign Exchange Contracts | Number of Contracts | Aggregate Notional Amount in Foreign Currency | Maturity |
|---|---|---|---|
| Euros – Purchase | 2 | 92.2 | July 2017 |
| British Pounds – Sell | 1 | 11.3 | July 2017 |
| Total | 3 | | |

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

| | Asset Derivatives June 30, 2017 | | Liability Derivatives June 30, 2017 | |
|---|---|---|---|---|
| | Balance Sheet Location | Fair Value | Balance Sheet Location | Fair Value |
| Derivatives designated as hedging instruments: | | | | |
| Forward foreign currency exchange contracts | Other current assets | $ 1,743 | Accounts payable and accrued liabilities | $ 9,434 |
| Forward foreign currency exchange contracts | Other assets | 2,558 | Other long- term liabilities | 6,506 |
| Total | | 4,301 | | 15,940 |
| Derivatives not designated as hedging instruments: | | | | |
| Forward foreign currency exchange contracts | Other current assets | 507 | Accounts payable and accrued liabilities | 58 |
| Total | | 507 | | 58 |
| Total value of derivative contracts | | $ 4,808 | | $ 15,998 |

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BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

| | Asset Derivatives December 31, 2016 | | Liability Derivatives December 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 | $ 13,048 | Accounts payable and accrued liabilities | $ 5,176 |
| Forward foreign currency exchange contracts | Other assets | 8,194 | Other long- term liabilities | 2,342 |
| Total | | 21,242 | | 7,518 |
| Derivatives not designated as hedging instruments: | | | | |
| Forward foreign currency exchange contracts | Other current assets | 964 | Accounts payable and accrued liabilities | 25 |
| Total | | 964 | | 25 |
| Total value of derivative contracts | | $ 22,206 | | $ 7,543 |

The effect of the Company's derivative instruments on the Condensed Consolidated Financial Statements for the three and six months ended June 30, 2017 and 2016 was as follows:

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2017 | 2016 | 2017 | 2016 |
| Derivatives Designated as Hedging Instruments: | | | | |
| Net gain (loss) recognized in accumulated other comprehensive income (loss) [1] | $(19,165) | $4,608 | $(23,364) | $(3,873) |
| Net gain (loss) reclassified from accumulated other comprehensive income (loss) into earnings [2] | 695 | (197) | 3,211 | 3,130 |
| Net gain (loss) recognized in net loss [3] | 826 | (603) | 1,706 | 5,267 |
| Derivatives Not Designated as Hedging Instruments: | | | | |
| Net gain (loss) recognized in net loss[4] | $5,373 | $1,012 | $5,631 | $(3,272) |

(1) Net change in the fair value of the effective portion classified as accumulated other comprehensive income (loss).
(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.

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 June 30, 2017 | | | |
|---|---|---|---|---|
| | Quoted Price in Active Markets For Identical Assets (Level 1) | Significant Other Observable Inputs (Level 2) | Significant Unobservable Inputs (Level 3) | Total |
| Assets: | | | | |
| Cash and cash equivalents: | | | | |
| Money market instruments | $— | $ 44,566 | $ — | $44,566 |
| Corporate debt securities | — | 1,709 | — | 1,709 |
| Total cash and cash equivalents | — | 46,275 | — | 46,275 |
| Available-for-sale securities: | | | | |
| Short-term: | | | | |
| Corporate debt securities | — | 252,696 | — | 252,696 |
| Commercial paper | — | 8,976 | — | 8,976 |
| U.S. government agency securities | — | 111,240 | — | 111,240 |
| Long-term: | | | | |
| Corporate debt securities | — | 360,045 | — | 360,045 |
| U.S. government agency securities | — | 121,822 | — | 121,822 |
| Greek government-issued bonds | — | 169 | — | 169 |
| Total available-for-sale securities | — | 854,948 | — | 854,948 |
| Other current assets: | | | | |
| NQDC Plan assets | — | 661 | — | 661 |
| Forward foreign currency exchange contract[1] | — | 2,250 | — | 2,250 |
| Restricted investments [2] | — | 9,327 | — | 9,327 |
| Total other current assets | — | 12,238 | — | 12,238 |

| | | | | |
|---|---|---|---|---|
| Other assets: | | | | |
| NQDC Plan assets | — | 11,347 | — | 11,347 |
| Forward foreign currency exchange contract(1) | — | 2,558 | — | 2,558 |
| Total other assets | — | 13,905 | — | 13,905 |
| Total assets | $— | $ 927,366 | $ — | $927,366 |
| Liabilities: | | | | |
| Current liabilities: | | | | |
| NQDC Plan liability | $2,326 | $ 661 | $ — | $2,987 |
| Forward foreign currency exchange contract (1) | — | 9,492 | — | 9,492 |
| Contingent acquisition consideration payable | — | — | 55,093 | 55,093 |
| Total current liabilities | 2,326 | 10,153 | 55,093 | 67,572 |
| Other long-term liabilities: | | | | |
| NQDC Plan liability | $17,844 | $ 11,347 | — | 29,191 |
| Forward foreign currency exchange contract (1) | — | 6,506 | — | 6,506 |
| Contingent acquisition consideration payable | — | — | 122,899 | 122,899 |
| Total other long-term liabilities | 17,844 | 17,853 | 122,899 | 158,596 |
| Total liabilities | $20,170 | $ 28,006 | $ 177,992 | $226,168 |

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, 2016 | | | |
|---|---|---|---|---|
| | Quoted Price in Active Markets For Identical Assets (Level 1) | Significant Other Observable Inputs (Level 2) | Significant Unobservable Inputs (Level 3) | Total |
| Assets: | | | | |
| Cash and cash equivalents: | | | | |
| Money market instruments | $— | $ 235,571 | $ — | $235,571 |
| Corporate debt securities | — | 8,593 | — | 8,593 |
| U.S. government agency securities | — | 6,000 | — | 6,000 |
| Total cash and cash equivalents | — | 250,164 | — | 250,164 |
| Available-for-sale securities: | | | | |
| Short-term: | | | | |
| Certificates of deposit | — | 2,800 | — | 2,800 |
| Corporate debt securities | — | 193,974 | — | 193,974 |
| Commercial paper | — | 16,075 | — | 16,075 |
| U.S. government agency securities | — | 168,498 | — | 168,498 |
| Long-term: | | | | |
| Corporate debt securities | — | 437,150 | — | 437,150 |
| U.S. government agency securities | — | 135,427 | — | 135,427 |
| Greek government-issued bonds | — | 134 | — | 134 |
| Total available-for-sale securities | — | 954,058 | — | 954,058 |
| Other current assets: | | | | |
| NQDC Plan assets | — | 163 | — | 163 |
| Forward foreign currency exchange contract[1] | — | 14,012 | — | 14,012 |
| Restricted investments [2] | — | 3,754 | — | 3,754 |
| Total other current assets | — | 17,929 | — | 17,929 |
| Other assets: | | | | |
| NQDC Plan assets | — | 9,121 | — | 9,121 |
| Forward foreign currency exchange contract [1] | — | 8,194 | — | 8,194 |
| Strategic investment [3] | 4,064 | — | — | 4,064 |
| Total other assets | 4,064 | 17,315 | — | 21,379 |
| Total assets | $4,064 | $ 1,239,466 | $ — | $1,243,530 |

| | | | | |
|---|---|---|---|---|
| Liabilities: | | | | |
| Current liabilities: | | | | |
| NQDC Plan liability | $2,073 | $ 163 | $ — | $2,236 |
| Forward foreign currency exchange contract(1) | — | 5,201 | — | 5,201 |
| Contingent acquisition consideration payable | — | — | 46,327 | 46,327 |
| Total current liabilities | 2,073 | 5,364 | 46,327 | 53,764 |
| Other long-term liabilities: | | | | |
| NQDC Plan liability | 17,303 | 9,121 | — | 26,424 |
| Forward foreign currency exchange contract (1) | — | 2,342 | — | 2,342 |
| Contingent acquisition consideration payable | — | — | 115,310 | 115,310 |
| Total other long-term liabilities | 17,303 | 11,463 | 115,310 | 144,076 |
| Total liabilities | $19,376 | $ 16,827 | $ 161,637 | $197,840 |

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

(2) The restricted investments at June 30, 2017 and December 31, 2016 secure the Company’s irrevocable standby letter of credit obtained in connection with certain commercial agreements.

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

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

There were no transfers between levels during the three and six months ended June 30, 2017.

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 6 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 Condensed Consolidated Statements of Comprehensive Loss. The probability-based income approach used by management to estimate the fair value of the contingent acquisition consideration is most sensitive to changes in the estimated probabilities.

| | |
|---|---|
| Contingent acquisition consideration payable at December 31, 2016 | $161,637 |
| Changes in the fair value of contingent acquisition consideration payable for | |
| continuing development programs | 7,195 |
| Milestone payments to former Huxley Pharmaceuticals, Inc. shareholders | (3,500) |
| Foreign exchange remeasurement of Euro denominated contingent | |
| acquisition consideration payable | 12,660 |
| Contingent acquisition consideration payable at June 30, 2017 | $177,992 |

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. As of June 30, 2017, the balance of the asset retirement obligation liability was $4.1 million.

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.

(14) STOCK-BASED COMPENSATION

The Company’s stock-based compensation plans include the 2017 Equity Incentive Plan (the 2017 Equity Incentive Plan) and the ESPP. The 2017 Equity Incentive Plan, which was approved by the Company’s stockholders on June 6, 2017 and became effective that same date, and is the successor to and continuation of the Company’s Amended and Restated 2006 Share Incentive Plan (the 2006 Share Incentive Plan), provides for awards of RSUs and stock options as well as other forms of equity compensation. No additional awards will be granted under the 2006 Share Incentive Plan; however, there are vested and unvested awards outstanding under the 2006 Share Incentive Plan. Stock option awards granted to employees generally vest over a four-year period on a cliff basis one year after the grant date and then monthly thereafter. The contractual term of the outstanding options is generally ten years. RSUs granted to employees generally vest annually on a straight-line basis over a four-year period after the grant date. RSUs granted to directors generally vest in full one year after the grant date. Shares formerly reserved for future issuance under the 2006 Share Incentive Plan

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were transferred to the 2017 Equity Incentive Plan, from which future shares shall be issued. The Company's stock-based compensation plans are administered by the Company's Board of Directors, or designated Committee thereof, 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.

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 are considered separately for valuation purposes. 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. Effective January 1, 2016, forfeitures were accounted for as they occurred. The assumptions used to estimate the per share fair value of stock options granted under the 2017 Equity Incentive Plan and the 2006 Share Incentive Plan were as follows:

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2017 | 2016 | 2017 | 2016 |
| Expected volatility | 38 – 40% | 37 - 40% | 38 – 40% | 36 – 44% |
| Dividend yield | 0.0% | 0.0% | 0.0% | 0.0% |
| Expected life | 5.0 – 6.6 years | 5.1 - 6.8 years | 5.0 – 6.6 years | 5.1 – 8.1 years |
| Risk-free interest rate | 1.8 – 1.9% | 1.1 - 1.5% | 1.8 – 2.2% | 1.1 – 2.1% |

During the six months ended June 30, 2017, the Company granted options to purchase 733,330 shares of common stock with a weighted average fair value of $36.02 per share.

The assumptions used to estimate the per share fair value of stock purchase rights granted under the ESPP were as follows:

| | Six Months Ended June 30, | |
|---|---|---|
| | 2017 | 2016 |
| Expected volatility | 31 - 42% | 42 - 50% |

| | | |
|---|---|---|
| Dividend yield | 0.0% | 0.0% |
| Expected life | 6-24 months | 6-24 months |
| Risk-free interest rate | 1.0 - 1.3% | 0.4 - 0.8% |

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, based on the market price of the Company's common stock on that date, ratably over the period during which the vesting restrictions lapse. During the six months ended June 30, 2017, the Company granted 1,230,410 RSUs with service-based vesting conditions with a weighted average fair value of $87.78 per share.

Restricted Stock Unit Awards with Performance Conditions

On March 22, 2017, pursuant to Board approval, the Company granted 133,250 RSUs with performance-vesting conditions (the 2017 Base RSUs) under the 2006 Share Incentive Plan to certain executive officers. The award of the RSUs under this specific grant is contingent upon the achievement of a 2017 revenue target and the awarded RSUs, if any, vest ratably over a three-year service period. The number of RSUs to be awarded upon achievement of the performance condition may range between 50% and 200% of the 2017 Base RSUs, dependent on the percentage of 2017 "managed revenues" (defined as the Company's net product revenues, excluding net revenues attributable to Aldurazyme) achieved against the target managed revenues with a threshold achievement level of 75% of target and a ceiling achievement level of 125% of target. 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

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2017 Base RSUs was determined to be $87.42 per RSU, based on the fair value of the common stock underlying the 2017 Base RSUs on the grant date based on the market price of the Company's common stock on that date. The Company evaluated the 2017 revenue target in the context of its current 2017 revenue forecast and related confidence level in the forecast, and determined that attainment of the revenue target was probable for accounting purposes commencing with the first quarter of 2017. As a result, the Company recognized $1.5 million and $1.6 million of compensation expense related to these awards during the three and six months ended June 30, 2017.

On March 15, 2016, pursuant to Board approval, the Company granted 130,310 RSUs with performance-vesting conditions (the 2016 Base RSUs) and a three-year service period, under the 2006 Share Incentive Plan, to certain executive officers. Based on the Company's performance against the 2016 revenue target, the Company applied a multiplier of 103% and issued 134,219 RSUs with a grant date fair value of $83.43 per RSU. The Company recognized $1.2 million and $2.3 million of compensation expense related to these awards during the three and six months ended June 30, 2017, respectively. The Company recognized $0.9 million and $1.1 million of compensation expense related to these awards during the three and six months ended June 30, 2016, respectively.

On March 3, 2015, pursuant to Board approval, the Company granted 58,300 RSUs with performance-vesting conditions (the 2015 Base RSUs) and a three-year service period, under the 2006 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 RSUs with a grant date fair value of $108.36 per RSU. The Company recognized $0.6 million and $1.2 million of compensation expense related to these awards during the three and six months ended June 30, 2017, respectively. The Company recognized $0.4 million and $1.2 million of compensation expense related to these awards during the three and six months ended June 30, 2016, 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 June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2017 | 2016 | 2017 | 2016 |
| Cost of sales | $2,510 | $2,289 | $4,796 | $3,851 |
| R&D | 14,647 | 14,814 | 26,141 | 28,521 |
| SG&A | 22,944 | 16,795 | 39,838 | 31,703 |
| Total stock-based compensation expense | $40,101 | $33,898 | $70,775 | $64,075 |

The Company adopted ASU No. 2016-09, Improvements to Employee Share-Based Payment Accounting, in 2016, noting that the impact of the election to use actual forfeitures rather than estimated forfeitures on quarterly reporting was insignificant.

Stock-based compensation expense of $4.4 million and $7.6 million was capitalized into inventory for the three and six months ended June 30, 2017 and Stock-based compensation expense of $3.3 million and $5.8 million was

capitalized into inventory for the three and six months ended June 30, 2016, respectively. Capitalized stock-based compensation is recognized as Cost of Sales when the related product is sold.

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NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

(15) ACCUMULATED OTHER COMPREHENSIVE INCOME (LOSS)

The following table summarizes amounts reclassified out of Accumulated Other Comprehensive Income (Loss) (AOCI) and their effect on the Company's Condensed Consolidated Statements of Comprehensive Loss for the three and six months ended June 30, 2017 and 2016.

| Details about AOCI Components | Three Months Ended June 30, 2017 | 2016 | Six Months Ended June 30, 2017 | 2016 | Consolidated Statement of Comprehensive Loss Classification |
|---|---|---|---|---|---|
| Gains (losses) on cash flow hedges: | | | | | |
| Forward foreign currency exchange contracts | $1,119 | $(589) | $4,661 | $2,600 | Net product revenues |
| Forward foreign currency exchange contracts | (424) | 392 | (1,450) | 4,824 | SG&A |
| Total gain (loss) on cash flow hedges | 695 | (197) | 3,211 | 7,424 | |
| Gain (loss) on sale of available-for-sale securities | — | (2,027) | 3,252 | (2,027) | Other income |
| Income tax effect of the above | — | 737 | (1,181) | 737 | Benefit from income taxes |
| | $695 | $(1,487) | $5,282 | $6,134 | Net loss |

The following tables summarize changes in the accumulated balances for each component of AOCI, including current period other comprehensive income (loss) and reclassifications out of AOCI for the three and six months ended June 30, 2017 and 2016.

| | Three Months Ended June 30, 2017 | | | |
|---|---|---|---|---|
| | Unrealized Gains (Losses) on Cash Flow Hedges | Unrealized Gains (Losses) on Available-for-Sale Securities | Foreign Currency Items | Total |
| AOCI balance at March 31, 2017 | $6,291 | $ (1,258) | $ (13) | $5,020 |
| Other comprehensive income (loss) before reclassifications | (19,165) | 267 | 4 | (18,894) |

| | | | | |
|---|---|---|---|---|
| Less net gain reclassified from AOCI | 695 | — | — | 695 |
| Tax effect | — | (89) | — | (89) |
| Net current-period other comprehensive loss | (19,860) | 178 | 4 | (19,678) |
| AOCI balance at June 30, 2017 | $(13,569) | $ (1,080) | $ (9) | $(14,658) |

| | Three Months Ended June 30, 2016 | | | |
|---|---|---|---|---|
| | Unrealized Gains (Losses) on Cash Flow Hedges | Unrealized Gains (Losses) on Available-for-Sale Securities | Foreign Currency Items | Total |
| AOCI balance at March 31, 2016 | $(2,500) | $ 2,554 | $ (7) | $47 |
| Other comprehensive income (loss) before reclassifications | 4,608 | (2,536) | (1) | 2,071 |
| Less loss reclassified from AOCI | (197) | (2,027) | — | (2,224) |
| Tax effect | — | 186 | — | 186 |
| Net current-period other comprehensive income (loss) | 4,805 | (323) | (1) | 4,481 |
| AOCI balance at June 30, 2016 | $2,305 | $ 2,231 | $ (8) | $4,528 |

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

| | Six Months Ended June 30, 2017 | | | |
|---|---|---|---|---|
| | Unrealized Gains (Losses) on Cash Flow Hedges | Unrealized Gains (Losses) on Available-for-Sale Securities | Foreign Currency Items | Total |
| AOCI balance at December 31, 2016 | $13,006 | $ (178) | $ (12) | $12,816 |
| Other comprehensive income (loss) before reclassifications | (23,364) | 1,834 | 3 | (21,527) |
| Less net gain reclassified from AOCI | 3,211 | 3,252 | — | 6,463 |
| Tax effect | — | 516 | — | 516 |
| Net current-period other comprehensive income (loss) | (26,575) | (902) | 3 | (27,474) |
| AOCI balance at June 30, 2017 | $(13,569) | $ (1,080) | $ (9) | $(14,658) |

| | Six Months Ended June 30, 2016 | | | |
|---|---|---|---|---|
| | Unrealized Gains (Losses) on Cash Flow Hedges | Unrealized Gains (Losses) 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 | (3,873) | (10,215) | 2 | (14,086) |
| Less gain (loss) reclassified from AOCI | 7,424 | (2,027) | — | 5,397 |
| Tax effect | — | 2,978 | — | 2,978 |
| Net current-period other comprehensive income (loss) | (11,297) | (5,210) | 2 | (16,505) |
| AOCI balance at June 30, 2016 | $2,305 | $ 2,231 | $ (8) | $4,528 |

(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 Brineura, Firdapse, Kuvan, Naglazyme, and Vimizim which are sold directly by the Company and global sales of Aldurazyme which is marketed by Genzyme. Genzyme is the Company's sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties.

| | Three Months Ended June 30, | | | | Six Months Ended June 30, | | | |
|---|---|---|---|---|---|---|---|---|
| | 2017 | | 2016 | | 2017 | | 2016 | |
| United States | 38 | % | 36 | % | 38 | % | 37 | % |
| Europe | 21 | % | 22 | % | 21 | % | 23 | % |
| Latin America | 18 | % | 17 | % | 15 | % | 13 | % |
| Rest of world | 17 | % | 19 | % | 20 | % | 20 | % |
| Total net product revenue marketed by the Company | 94 | % | 94 | % | 94 | % | 93 | % |
| Aldurazyme net product revenues marketed by Genzyme | 6 | % | 6 | % | 6 | % | 7 | % |
| Total net product revenues | 100 | % | 100 | % | 100 | % | 100 | % |

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(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 largest customers for the three and six months ended June 30, 2017 and 2016.

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2017 | 2016 | 2017 | 2016 |
| Customer A | 17% | 19 % | 17% | 19 % |
| Customer B | 14% | 12 % | 13% | 13 % |
| Customer C | 10% | 9 % | 10% | 10 % |
| Customer D | 10% | 10 % | 7 % | 6 % |
| Total | 51% | 50 % | 47% | 48 % |

On a consolidated basis, the Company's two largest customer accounts receivable balances accounted for 21% and 19% of the June 30, 2017 total accounts receivable balance, respectively, compared to December 31, 2016, when the two largest customer accounts receivable balances accounted for 26% and 20% of the total accounts receivable balance, respectively. As of June 30, 2017, and December 31, 2016, the accounts receivable balance for Genzyme included $20.0 million and $30.7 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 sells its products in countries, including Southern European countries, Russia, Chile and Brazil, which 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 in these countries. The Company believes that the allowances for doubtful accounts related to these countries, if any, is adequate based on its analysis of the specific business circumstances and expectations of collection for each of the underlying accounts in these countries.

(17) SEGMENT INFORMATION

The Company operates in one business segment, which focuses on the development and commercialization of innovative therapies for people with serious and life threatening rare diseases and medical conditions. All products are included in one segment because all 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 June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2017 | 2016 | 2017 | 2016 |
| Net product revenues by product: | | | | |
| Aldurazyme | $19,985 | $18,623 | $39,340 | $35,068 |
| Brineura | 254 | — | 254 | — |
| Firdapse | 4,855 | 4,465 | 8,965 | 8,704 |
| Kuvan | 101,944 | 90,215 | 194,290 | 166,907 |
| Naglazyme | 85,751 | 78,444 | 166,309 | 143,847 |
| Vimizim | 103,137 | 106,829 | 208,958 | 179,407 |
| Total net product revenues | $315,926 | $298,576 | $618,116 | $533,933 |

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

The following table summarizes total revenues from external customers and collaborative partners by geographic region. Net product revenues by geographic region are based on patient location for the Company's commercial products, except for Aldurazyme, which is based on the location of Genzyme's headquarters. Although Genzyme sells Aldurazyme worldwide, the revenues earned by the Company based on Genzyme's net sales are included in the U.S. region, as the transactions are with Genzyme whose headquarters are located in the U.S.

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2017 | 2016 | 2017 | 2016 |
| Total revenues by geographic region: | | | | |
| United States | $141,612 | $126,172 | $274,097 | $235,318 |
| Europe | 64,931 | 64,798 | 130,950 | 124,508 |
| Latin America | 58,205 | 49,635 | 95,468 | 68,015 |
| Rest of world | 52,700 | 59,526 | 120,678 | 109,026 |
| Total revenues | $317,448 | $300,131 | $621,193 | $536,867 |

(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.

Paragraph IV Notices

The Company received a paragraph IV notice letter, dated October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying us that DRL 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 FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Together with Merck & Cie, on November 17, 2014, the Company filed a lawsuit against DRL in the U.S. District Court for the District of New Jersey alleging infringement of the Company's patents relating to Kuvan tablets and seeking an injunction to prevent DRL from introducing a generic version of Kuvan tablets that would infringe the Company's patents prior to their expiration. In September 2015, the

Company and Merck & Cie entered into a settlement agreement with DRL (the DRL Tablet Settlement Agreement) that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral tablets. Under the terms of the DRL Tablet Settlement Agreement, the Company granted DRL a non-exclusive license to its Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg oral tablets in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances.

Additionally, the Company received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying it that Par had filed an ANDA seeking approval of a proposed generic version of Kuvan 100 mg oral tablets prior to the expiration of the Company's patents listed in the FDA's 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.

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

The Company also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying it that Par had 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.

In April 2017, the Company and Merck & Cie entered into a settlement agreement with Par (the Par Settlement Agreement) that resolved both cases against Par. Under the Par Settlement Agreement, the Company granted Par a non-exclusive license to its Kuvan-related patents to allow Par to market a generic version of sapropterin dihydrochloride in 100 mg oral tablets and oral powder in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on: April 1, 2021 if Par is not entitled to the statutory 180-day first filer exclusivity period; October 1, 2020 if Par is entitled to the statutory 180-day first filer exclusivity period; or earlier under certain circumstances.

The Company also received a paragraph IV notice letter, dated December 23, 2016, from DRL, notifying it that DRL had 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 6, 2017, the Company filed a lawsuit against DRL 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 DRL from introducing a generic version of Kuvan powder that would infringe the Company's patents prior to their expiration. The filing of that lawsuit triggered the automatic 30-month stay on the approval of DRL's ANDA in accordance with the Hatch-Waxman Act, which expires in June 2019 or earlier if the court enters judgment finding that each of the then-asserted patents is invalid or not infringed, or such shorter or longer period as the court may order. DRL filed its answer to the complaint on April 10, 2017, alleging, inter alia, that the patents are not infringed and/or are invalid. Neither the DRL Tablet Settlement Agreement nor the Par Settlement Agreement affects this currently pending litigation against DRL relating to Kuvan 100 mg oral powder.

SEC Subpoena

In August 2016, the Company received a subpoena from the staff of the SEC requesting that the Company produce documents in connection with a non-public, fact-finding inquiry related to its former drisapersen program. The letter enclosing the subpoena states that the investigation and the subpoena do not mean that the Company or anyone else has broken the law, or that the SEC has a negative opinion of any person, entity or security. The Company intends to cooperate fully with the SEC in this matter. The Company is not able to predict whether any proceeding may be instituted in connection with the subpoena, or the outcome of any proceeding that may be instituted.

Contingent Payments

As of June 30, 2017, the Company is subject to contingent payments totaling approximately $585.5 million upon achievement of certain development and regulatory activities and commercial sales and licensing milestones if they

occur before certain dates in the future. Of this amount, $210.9 million (or €185 million based on the exchange rate of 1.14 USD per Euro in effect on June 30, 2017) relates to the Merck PKU Business acquisition and $52.2 million relates to programs that are no longer being developed.

As of June 30, 2017, the Company has recorded a total of $178.0 million of short-term and long-term contingent acquisition consideration payable on its Condensed Consolidated Balances Sheet, of which $55.1 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 June 30, 2017, these commitments for the next five years were approximately $73.8 million. The amounts primarily represent minimum purchase requirements for active pharmaceutical ingredients and post-marketing commitments related to the Company’s approved products.

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NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS – (Continued)

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

## (19) BENEFIT FROM INCOME TAXES

The Company has historically computed its interim period 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 can be highly sensitive to minor fluctuations in U.S. forecasted income. As such, the Company has computed the U.S. component of the consolidated benefit from income taxes for the three and six months ended June 30, 2017 and 2016 using an actual year-to-date tax calculation. Foreign tax expense was computed using a forecasted annual effective tax rate for the three and six months ended June 30, 2017 and 2016.

## (20) SUBSEQUENT EVENT

On July 17, 2017, the Company executed license and settlement agreements (the Agreements) with Sarepta Therapeutics (Sarepta) that provide Sarepta with global exclusive rights to the Company’s Duchenne muscular dystrophy (DMD) patent estate for EXONDYS 51 and all future exon-skipping products. The Agreements resolved the ongoing worldwide patent proceedings related to the use of EXONDYS 51 and all future exon-skipping products for the treatment of DMD. Under the Agreements, Sarepta made a one-time $35.0 million payment to the Company and certain additional regulatory and commercial milestone payments for exons 51, 45, 53 and possibly on future exon-skipping products to the Company. Sarepta will also pay royalties to the Company based on 5% of net sales through the end of 2023 in the U.S. and 8% of net sales through September 30, 2024 in the EU and in other countries where certain of the Company’s patents exist. The Company retained the right to convert the license to a co-exclusive right in the event it decides to proceed with an exon-skipping therapy for DMD.

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

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 in this Quarterly Report on Form 10-Q. This discussion contains forward-looking statements that involve risks and uncertainties. When reviewing the discussion below, you should keep in mind the substantial risks and uncertainties that could impact our business. In particular, we encourage you to review the risks and uncertainties described in "Risk Factors" in Part II, Item 1A in this Quarterly Report on Form 10-Q. These risks and uncertainties could cause actual results to differ significantly from those projected in forward-looking statements contained in this report or implied by past results and trends. Forward-looking statements are statements that attempt to forecast or anticipate future developments in our business, financial condition or results of operations. See the section titled "Forward-Looking Statements" that appears at the beginning of this Quarterly Report on Form 10-Q. These statements, like all statements in this report, speak only as of the date of this Quarterly Report on Form 10-Q (unless another date is indicated), and, except as required by law, we undertake no obligation to update or revise these statements in light of future developments.

Overview

We are a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare 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 therapy portfolio consists of six products and multiple clinical and pre-clinical product candidates. Our commercial products are Aldurazyme (laronidase) for Mucopolysaccharidosis I (MPS I), Brineura (cerliponase alfa) for the treatment of late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), Firdapse (amifampridine phosphate) for Lambert Eaton Myasthenic Syndrome (LEMS), Kuvan (sapropterin dihydrochloride) for phenylketonuria (PKU), Naglazyme (galsulfase) for Mucopolysaccharidosis VI (MPS VI) and Vimizim (elosulfase alpha) for Mucopolysaccharidosis IV Type A (MPS IV A).

Business Developments

We continued to grow our commercial business and advance our product pipeline during the first half of 2017. We believe that the combination of our internal research programs, acquisitions and partnerships will allow us to continue to develop and commercialize innovative therapies for people with serious and life-threatening rare diseases and medical conditions. Below is a summary of key business developments in 2017 to date:

- In April 2017, the FDA approved Brineura, the first treatment approved to slow the progression of loss of ambulation children with CLN2 disease. We immediately began marketing Brineura in the U.S., and began shipping the product within the U.S. in mid-June 2017.
- In June 2017, we announced that the European Commission granted marketing authorization for Brineura in the European Union (EU) to treat children with CLN2 disease. Brineura is the first treatment approved in the EU for the treatment of CLN2 disease and the marketing authorization for Brineura includes all 28 countries of the EU, Norway, Iceland and Liechtenstein. We immediately began marketing Brineura in the EU and began shipping the product within the EU in July 2017.
- In July 2017, subsequent to the second quarter, we announced the execution of a license agreement and a settlement agreement (the Agreements) with Sarepta Therapeutics (Sarepta) that provide Sarepta with global exclusive rights to the Company's Duchenne muscular dystrophy (DMD) patent estate for EXONDYS 51 and all future exon-skipping products. The Agreements resolved the ongoing worldwide patent proceedings related to the use of EXONDYS 51 and all future exon-skipping products for the treatment of DMD. Under the Agreements, Sarepta will make a one-time $35.0 million payment to the Company and certain additional regulatory and commercial milestone payments for exons 51, 45, 53 and possibly on future exon-skipping products to the Company. Sarepta will also pay royalties to us based on 5% of net sales through the end of 2023 in the U.S. and 8% of net sales through September

30, 2024 in the EU and in other countries where certain of the Company's patents exist. The Company retained the right to convert the license to a co-exclusive right in the event it decides to proceed with an exon-skipping therapy for DMD.

- In June 2017, we submitted a Biologics License Application (BLA) to the FDA for pegvaliase, a PEGylated phenylanine-metabolizing enzyme product, to reduce blood phenylalanine (Phe) levels in adult patients with PKU who have uncontrolled blood Phe levels greater than 600 µmol/L on existing management. Following receipt of the BLA, the FDA conducts an initial assessment of the application to determine whether to accept it for substantive review. We expect to hear from FDA within approximately two months of submission as to whether FDA is going to accept the application for substantive review. We also intend to submit an application for registration in the EU by the end of 2017.

- In July 2017, we announced that we will expand our development plan for BMN 270, our investigational gene therapy for Hemophilia A, to include an additional Phase 3 study of the 4e13 vg/kg dose based on updated data as of July 28, 2017 from our ongoing open-label Phase 1/2 study of BMN 270. Since the last data update presented, the Factor VIII activity levels in the 4e13 vg/kg cohort have continued to trend upwards and now support an additional Phase 3 study to the development program. Based on the most recent data, for the three patients who were given the 4e13 vg/kg dose in November/December 2016, at week 32, all are in or near to the normal range of Factor VIII activity levels, with both median and mean Factor VIII levels of 51%. For the cohort of three patients who were given the 4e13 vg/kg dose in February/March 2017, at week 20, their Factor VIII activity levels have all moved into the mild range and two of the three are continuing to trend upward. For all six patients who received a dose of 4e13 vg/kg, at week 20, the median Factor VIII level was 34% and the mean was 31%. Based on these updated results, we plan to initiate two separate Phase 3 studies, one with the 4e13 vg/kg dose and one with the 6e13 vg/kg dose. In July 2017, we announced that all patients at the 6e13 vg/kg dose had reached 52 weeks of post-treatment follow-up. Median and mean Factor VIII levels from week 20 through 52 for the 6e13 vg/kg dose cohort have been consistently within the normal levels post treatment. At one year after dosing, the median and mean Factor VIII levels of the 6e13 vg/kg cohort continue to be above 50%. In addition, we announced that we have commissioned our commercial gene therapy manufacturing facility and expect to start the Phase 3 program in the fourth quarter of 2017.
- In December 2016, we announced the enrollment of the first patient in our Phase 3 trial for vosoritide, for the treatment of children with achondroplasia. The Phase 3 study is a randomized, placebo-controlled study of vosoritide in approximately 110 children with achondroplasia ages 5-14 for 52 weeks. The study will be followed by a subsequent open-label extension. In April 2017, following discussions with global health authorities, we announced plans to augment the growth velocity data in the Phase 3 study with assessments of proportionality, functionality and cumulative growth observed in that study and the ongoing Phase 2 study, as well as safety and efficacy in infants.
- In April 2017, we announced that we entered into a settlement agreement with Par Pharmaceutical (Par) that resolves patent litigation in the U.S. related to our Kuvan (sapropterin dihydrochloride) 100 mg oral tablets and powder for oral solution in 100 mg packets. Under the terms of the settlement, we will grant Par a non-exclusive license to its patents related to Kuvan to allow Par to market a generic version of sapropterin dihydrochloride 100 mg tablets and powder for oral solution in 100 mg and 500 mg sachets in the U.S. for the indications approved for Kuvan beginning on: April 1, 2021 if Par is not entitled to the statutory 180-day first filer exclusivity period; October 1, 2020 if Par is entitled to the statutory 180-day first filer exclusivity period; or earlier under certain circumstances. The settlement with Par does not affect pending litigation against Dr. Reddy’s Laboratories, Inc. and Dr. Reddy’s Laboratories, Ltd. (collectively, DRL) relating to Kuvan 100 mg oral powder.
- In January 2017, we announced preliminary results from a Phase 1/2 trial of BMN 250, an investigational enzyme replacement therapy using a novel fusion of recombinant human NAGLU with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome, or MPS IIIB, which began enrolling patients in April 2016. We observed that BMN 250 reduced heparan sulfate levels to normal range in cerebral spinal fluid of MPS IIIB patients.
- We reported total revenues of $317.4 million $621.2 million for the three and six months ended June 30, 2017, respectively, as compared to $300.1 million and $536.9 million for the three and six months ended June 30, 2016, respectively.

Financial Highlights

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

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2017 | 2016 | 2017 | 2016 |

| | | | | |
|---|---|---|---|---|
| Total revenues | $317.4 | $300.1 | $621.2 | $536.9 |
| Cost of sales | 56.3 | 51.6 | 106.3 | 94.7 |
| Research and Development (R&D) expense | 143.0 | 167.0 | 288.0 | 325.8 |
| Selling, general and administrative (SG&A) expense | 143.5 | 109.6 | 263.5 | 214.9 |
| Intangible asset amortization and contingent consideration expense | 13.4 | (54.4) | 22.3 | (44.0) |
| Impairment of intangible assets | — | 599.1 | — | 599.1 |
| Net loss | (36.8) | (419.0) | (53.1) | (502.1) |
| Stock-based compensation expense | 40.1 | 33.9 | 70.8 | 64.1 |

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

Total revenues include net product revenues and royalty and other revenues. Net product revenues are generated from the six approved products in our product portfolio as of June 30, 2017. In the U.S., our commercial products are generally sold to specialty pharmacies or end-users, such as hospitals, which act as retailers. Outside the U.S., our commercial products are sold to our authorized distributors or directly to government purchasers or hospitals, which act as the end-users. Royalty and other revenues include royalties on net sales of products to licensees or sublicensees, collaborative agreement revenues and rental income associated with the tenants in our San Rafael, California facility.

Cost of sales includes raw materials, personnel and facility and other costs associated primarily with manufacturing Aldurazyme, Brineura, Naglazyme and Vimizim 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.

Our cash, cash equivalents, short-term investments and long-term investments totaled $1.2 billion as of June 30, 2017, compared to $1.4 billion as of December 31, 2016. We have historically financed our operations primarily through our cash flows from operating activities and the issuance of common stock and convertible debt. We expect to fund our operations with our net product revenues from our commercial products, cash, cash equivalents, and short-term and long-term investments, supplemented by proceeds from equity or debt financings and loans, or collaborative agreements with corporate partners. The timing and mix of our funding options could change depending on many factors, including 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. 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 GAAP in the U.S. and pursuant to the rules and regulations promulgated by the SEC, we make assumptions, judgments and estimates that can have a significant impact on our net income/loss and affect the reported amounts of certain assets, liabilities, revenue and expenses, and related disclosures. We base our assumptions, judgments and estimates on historical experience and various other factors that we believe to be reasonable under the circumstances. Actual results could differ materially from these estimates under different assumptions or conditions.

On a regular basis, we evaluate our assumptions, judgments and estimates. We also discuss our critical accounting policies and estimates with the Audit Committee of our Board of Directors.

We believe that the assumptions, judgments and estimates involved in the accounting for business combinations, contingent acquisition consideration payable, income taxes, long-lived assets and revenue recognition 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 six months ended June 30, 2017, 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, 2016.

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 June 30, 2017 was $36.8 million, compared to a net loss of $419.0 million for the three months ended June 30, 2016. Our net loss for the six months ended June 30, 2017, was $53.1 million, compared to a net loss of $502.1 million for the six months ended June 30, 2016. The decrease in net loss was primarily a result of the following (in millions):

| | Three Months Ended June 30, | | | Six Months Ended June 30, | | |
|---|---|---|---|---|---|---|
| | 2017 | 2016 | Change | 2017 | 2016 | Change |
| Total revenues | $317.4 | $300.1 | $17.3 | $621.2 | $536.9 | $84.3 |
| Cost of sales | 56.3 | 51.6 | 4.7 | 106.3 | 94.7 | 11.6 |
| R&D expense | 143.0 | 167.0 | (24.0) | 288.0 | 325.8 | (37.8) |
| SG&A expense | 143.5 | 109.6 | 33.9 | 263.5 | 214.9 | 48.6 |
| Intangible asset amortization and contingent consideration expense | 13.4 | (54.4) | 67.8 | 22.3 | (44.0) | 66.3 |
| Impairment of intangible assets | — | 599.1 | (599.1) | — | 599.1 | (599.1) |
| Other, net [1] | (6.7) | (10.1) | 3.4 | (10.9) | (18.5) | 7.6 |
| Benefit from income taxes | (8.7) | (163.9) | 155.2 | (16.7) | (170.0) | 153.3 |
| Net loss | $(36.8) | $(419.0) | $382.2 | $(53.1) | $(502.1) | $449.0 |

(1) Includes Equity in the loss of BioMarin/Genzyme LLC, interest income, interest expense and other income (expense).

The decrease in net loss for the three and six months ended June 30, 2017 was primarily attributed to a $599.1 million impairment charge during the three months ended June 30, 2016, partially offset by a related decrease in the benefit from income taxes. See below for additional information related to the net loss fluctuations presented above, including details of our operating expense fluctuations and the aforementioned impairment charge.

Net Product Revenues

A summary of our various commercial products, including key metrics as of June 30, 2017, is provided below:

| Commercial Products | Indication | U.S. Orphan Drug Exclusivity Expiration | U.S. Biologic Exclusivity Expiration | EU Orphan Drug Exclusivity Expiration |
|---|---|---|---|---|
| Aldurazyme | MPS I | Expired | Expired | Expired |
| Brineura | CLN2 | 2024 | 2029 | 2027 |
| Firdapse | LEMS | NA [1] | NA | 2019 |
| Kuvan | PKU | Expired | NA | 2020 [2] |
| Naglazyme | MPS VI | Expired | 2017 | Expired |
| Vimizim | MPS IVA | 2021 | 2026 | 2024 |

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

(2) Kuvan has been granted orphan drug status in the EU, which together with pediatric exclusivity, confers 12 years of market exclusivity in the EU that expires in 2020. Furthermore, Merck Serono marketed Kuvan in the EU until January 1, 2016. See Note 5 to our accompanying Condensed Consolidated Financial for further discussion.

Net product revenues consisted of the following (in millions):

| | Three Months Ended June 30, | | | Six Months Ended June 30, | | |
|---|---|---|---|---|---|---|
| | 2017 | 2016 | Change | 2017 | 2016 | Change |
| Aldurazyme | $19.9 | $18.7 | $ 1.2 | $39.3 | $35.1 | $ 4.2 |
| Brineura | 0.3 | — | 0.3 | 0.3 | — | 0.3 |
| Firdapse | 4.8 | 4.5 | 0.3 | 8.9 | 8.7 | 0.2 |
| Kuvan | 102.0 | 90.2 | 11.8 | 194.3 | 166.9 | 27.4 |
| Naglazyme | 85.7 | 78.4 | 7.3 | 166.3 | 143.8 | 22.5 |
| Vimizim | 103.2 | 106.8 | (3.6) | 209.0 | 179.4 | 29.6 |
| Total net product revenues | $315.9 | $298.6 | $ 17.3 | $618.1 | $533.9 | $ 84.2 |

Our Firdapse, Kuvan, Naglazyme and Vimizim customers include a limited number of specialty pharmacies and end-users, such as hospitals and foreign government agencies. We also sell Brineura, Kuvan, Naglazyme and Vimizim to our authorized distributors and to certain larger pharmaceutical wholesalers globally, which act as intermediaries between us and end-users and generally do not stock significant quantities of our products. However, in certain countries, particularly in Latin America, governments place large periodic orders for Naglazyme and Vimizim. The timing of these large government orders can be inconsistent and can create significant quarter to quarter variation in our revenues. Genzyme is our sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third parties.

We face exposure to movements in foreign currency exchange rates, primarily the Euro. We use foreign currency exchange contracts to hedge a percentage of our foreign currency exposure. The following table shows our net product revenues denominated in U.S. dollar (USD) and foreign currencies (in millions):

| | For the Three Months Ended June 30, | | | For the Six Months Ended June 30, | | |
|---|---|---|---|---|---|---|
| | 2017 | 2016 | Change | 2017 | 2016 | Change |
| Sales denominated in USD | $187.3 | $171.4 | $ 15.9 | $366.5 | $305.6 | $ 60.9 |
| Sales denominated in foreign currencies | 128.6 | 127.2 | 1.4 | 251.6 | 228.3 | 23.3 |
| Total net product revenues | $315.9 | $298.6 | $ 17.3 | $618.1 | $533.9 | $ 84.2 |

The net impact of foreign currency exchange rates on product sales denominated in currencies other than USD during the three and six months ended June 30, 2017 was positive by $1.5 million and $2.4 million, respectively, compared to a positive impact of $1.3 million and $4.1 million, respectively, during the three and six months ended June 30, 2016.

The following is additional discussion of our revenue results by product:

- Aldurazyme: The increase in Aldurazyme net product revenues for the three and six months ended June 30, 2017, compared to the three and six months ended June 30, 2016, was primarily attributable to increased Aldurazyme revenues reported by Genzyme, offset in part by a decrease in shipments to Genzyme. Aldurazyme revenues reported by Genzyme totaled $62.4 million and $117.9 million for the three and six months ended June 30, 2017, respectively compared to $56.8 million and $109.6 million for the three and six months ended June 30, 2016, respectively. Although Genzyme sells Aldurazyme worldwide, the net product revenues earned by us on Genzyme's net sales are denominated in USD.
    - Brineura: We obtained FDA approval in April 2017 and immediately began marketing Brineura in the U.S. Shipments of Brineura within the U.S. commenced in mid-June 2017.
- Kuvan: The increase in Kuvan net product revenues for the three and six months ended June 30, 2017, compared to the three and six months ended June 30, 2016, was primarily attributable to an increase in patients on Kuvan therapy in the U.S. and the completion of the transition of the ex-North American territories acquired in 2016.
- Naglazyme: The increase in Naglazyme net product revenues for the three and six months ended June 30, 2017, compared to the three and six months ended June 30, 2016, was primarily attributable to the timing of central government orders from Latin America and the Middle East.
- Vimizim: The increase in Vimizim net product revenues for the three and six months ended June 30, 2017, compared to the three and six months ended June 30, 2016, was primarily attributed to the timing of orders and new patients initiating therapy.

Cost of Sales and Product Gross Margin

The following table summarizes our cost of goods sold and product gross margin (in millions, except percentages):

| | For the Three Months Ended June 30, | | | For the Six Months Ended June 30, | | |
|---|---|---|---|---|---|---|
| | 2017 | 2016 | Change | 2017 | 2016 | Change |
| Total net product sales | $315.9 | $298.6 | $ 17.3 | $618.1 | $533.9 | $ 84.2 |
| Cost of sales | 56.3 | 51.6 | 4.7 | 106.3 | 94.7 | 11.6 |
| Product gross margin | 82 % | 83 % | -1 % | 83 % | 82 % | 1 % |

Product gross margin (net product revenues less cost of sales, expressed as a percentage of net product revenues) for the three and six months ended June 30, 2017 as compared to 2016 remained relatively flat.

Research and Development

A summary of our on-going major development programs, including key metrics as of June 30, 2017, is provided below:

| Major Products in Development | Target Indication | U.S. Orphan Designation | EU Orphan Designation | Stage |
|---|---|---|---|---|
| BMN 250 | MPS IIIB | Yes | Yes | Clinical Phase 1/2 |
| BMN 270 | Hemophilia A | Yes | Yes | Clinical Phase 1/2 |
| Pegvaliase | PKU | Yes | Yes | Marketing authorization<br><br>regulatory review |
| Vosoritide | Achondroplasia | Yes | Yes | Clinical Phase 3 |

We manage our R&D expense by identifying the R&D 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 decreased to $143.0 million and $288.0 million for the three and six months ended June 30, 2017, respectively, from $167.0 million and $325.8 million for the three and six months ended June 30, 2016, respectively. R&D expense consisted of the following (in millions):

| | Three Months Ended June 30, | | | Six Months Ended June 30, | | |
|---|---|---|---|---|---|---|
| | 2017 | 2016 | Change | 2017 | 2016 | Change |
| BMN 250 | $11.4 | $11.5 | $(0.1) | $20.9 | $23.9 | $(3.0) |
| BMN 270 | 32.6 | 14.4 | 18.2 | 53.9 | 26.1 | 27.8 |
| Brineura | 12.1 | 16.2 | (4.1) | 28.7 | 32.2 | (3.5) |
| Pegvaliase | 30.6 | 20.5 | 10.1 | 56.4 | 39.5 | 16.9 |
| Vosoritide | 12.3 | 10.8 | 1.5 | 25.9 | 23.9 | 2.0 |
| Other approved products | 20.1 | 15.7 | 4.4 | 37.3 | 33.7 | 3.6 |
| Early stage programs | 11.8 | 12.6 | (0.8) | 31.4 | 24.7 | 6.7 |
| Other and non-allocated | 12.1 | 65.3 | (53.2) | 33.5 | 121.8 | (88.3) |
| Total | $143.0 | $167.0 | $(24.0) | $288.0 | $325.8 | $(37.8) |

For the three and six months ended June 30, 2017, the decrease of $24.0 million and $37.8 million in R&D expense, respectively, as compared to the same period in 2016, was primarily related to the following:

- a decrease in R&D expense for other and non-allocated programs is primarily related to R&D spending in 2016 on the Kyndrisa, other exons, and reveglucosidase alfa development programs, all of which were terminated in 2016; and
- a decrease in R&D expense related to Brineura due to the capitalization of costs into inventory, which commenced in the second quarter of 2016; offset by

- an increase in clinical trial activities related to BMN 270 and pegvaliase product candidates; and
- an increase in early stage programs in the six month comparative was primarily attributable to the pre-clinical activity.

During the remainder of 2017, we expect our R&D spending to increase over 2016 levels due to our BMN 250, BMN 270, pegvaliase and vosoritide programs progressing in their development. We also expect increased spending on pre-clinical activities for our early development stage 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 or pre-qualification 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 or pre-qualification manufacturing activities for purposes of commercial sales will likely 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 $143.5 million and $263.5 million for the three and six months ended June 30, 2017, respectively, from $109.6 million and $214.9 million for the three and six months ended June 30, 2016, respectively. The increase in SG&A expense was primarily a result of the following (in millions):

| | Three Months Ended June 30, | | | Six Months Ended June 30, | | |
|---|---|---|---|---|---|---|
| | 2017 | 2016 | Change | 2017 | 2016 | Change |
| Sales and marketing (S&M) expense | $72.1 | $59.9 | $ 12.2 | $134.9 | $113.6 | $ 21.3 |
| General and administrative (G&A) expense | 71.4 | 49.7 | 21.7 | 128.6 | 101.3 | 27.3 |
| Total SG&A expense | $143.5 | $109.6 | $ 33.9 | $263.5 | $214.9 | $ 48.6 |

| | Three Months Ended June 30, | | | Six Months Ended June 30, | | |
|---|---|---|---|---|---|---|
| S&M expense by product | 2017 | 2016 | Change | 2017 | 2016 | Change |
| Brineura | $7.6 | $2.3 | $ 5.3 | $12.4 | $4.3 | $ 8.1 |
| Kuvan | 23.4 | 14.8 | 8.6 | 43.2 | 29.3 | 13.9 |
| Naglazyme | 13.6 | 12.5 | 1.1 | 25.4 | 24.0 | 1.4 |
| Vimizim | 17.7 | 16.8 | 0.9 | 35.3 | 31.1 | 4.2 |
| Other and not allocated | 9.8 | 13.5 | (3.7) | 18.6 | 24.9 | (6.3) |
| Total S&M expense | $72.1 | $59.9 | $ 12.2 | $134.9 | $113.6 | $ 21.3 |

S&M expense primarily consisted of employee-related expenses for our sales group, brand marketing, patient support groups and pre-commercialization expenses related to our product candidates. For the three and six months ended June 30, 2017, the increase of $12.2 million and $21.3 million in S&M expense, respectively, as compared to the same period in 2016, was primarily related to the following:

- an increase in Kuvan S&M expense due to continued worldwide expansion of commercial activities as a result of acquiring the worldwide rights to Kuvan, except for Japan, on January 1, 2016;
- an increase in Brineura S&M expense primarily due to the launch of commercial marketing expense;
- an increase in Vimizim S&M expense due to continued expansion of our worldwide commercial activities; offset by
- a decrease in other and not allocated S&M expense primarily due to the decrease in S&M expenses related to the terminated programs, including Kyndrisa and other exon programs.

G&A expense primarily consisted of corporate support and other administrative expenses, including employee-related expenses, which increased in the three and six months ended June 30, 2017, as compared to the three and six months ended June 30, 2016, primarily due to increased personnel and related costs.

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

Intangible Asset Amortization and Contingent Consideration

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 June 30, | | | Six Months Ended June 30, | | |
|---|---|---|---|---|---|---|
| | 2017 | 2016 | Change | 2017 | 2016 | Change |
| Changes in the fair value of contingent acquisition consideration payable | $5.8 | $(62.0) | $ 67.8 | $7.2 | $(59.1) | $ 66.3 |
| Amortization of intangible assets | 7.6 | 7.6 | — | 15.1 | 15.1 | — |
| Total intangible asset amortization and contingent consideration | $13.4 | $(54.4) | $ 67.8 | $22.3 | $(44.0) | $ 66.3 |

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 and six months ended June 30, 2017, the majority of the changes related to the progress of the PKU developmental program for pegvaliase. During the three and six months ended June 30, 2016, the majority of the changes related to the termination of the Kyndrisa and reveglucosidase alfa development programs that resulted in the reversal of the fair value of the remaining contingent consideration payable to the former Prosensa and Zystor Therapeutics, Inc. shareholders. The remaining continent consideration payable related to milestones payable upon the achievement of certain Kyndrisa and reveglucosidase alfa development, regulatory and sales milestones will not be attained now that the internal development of the programs has been terminated.

Impairment of Intangible Assets

In the second quarter of 2016, we recorded an impairment charge of $599.1 million related to the Kyndrisa and other exon and reveglucosidase alfa IPR&D assets based on the termination of the internal development of the respective programs. No impairment charges were recorded in the three and six months ended June 30, 2017. See Note 8 to our accompanying Condensed Consolidated Financial Statements for additional information regarding our Intangible Assets.

Interest Income

We invest our cash, short-term and long-term investments in U.S. government securities and other high credit quality securities in order to limit default and market risk. Interest income totaled $3.0 million and $6.1 million for the three and six months ended June 30, 2017, respectively, compared to $1.4 million and $2.9 million for the three and six months ended June 30, 2016, respectively. The increase in interest income during the three and six months ended June 30, 2017, as compared to the three and six months ended June 30, 2016 was primarily due to higher investment balances, which increased due to the August 2016 public offering of our common stock, and higher average interest rate on investments. Due to higher interest rates and planned spend, we do not expect interest income to fluctuate significantly over the next 12 months.

Interest Expense

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

| | Three Months Ended June 30, | | | Six Months Ended June 30, | | |
|---|---|---|---|---|---|---|
| | 2017 | 2016 | Change | 2017 | 2016 | Change |
| Coupon interest | $2.1 | $2.5 | $ (0.4) | $4.6 | $5.0 | $ (0.4) |
| Amortization of issuance costs | 0.9 | 0.8 | 0.1 | 1.8 | 1.7 | 0.1 |
| Accretion of discount on convertible notes | 7.0 | 6.6 | 0.4 | 13.8 | 13.1 | 0.7 |
| Total interest expense | $10.0 | $9.9 | $ 0.1 | $20.2 | $19.8 | $ 0.4 |

Interest expense primarily consisted of amounts related to our October 2013 issuance of $750.0 million in aggregate principal amount of senior subordinated convertible debt, including $375.0 million principal amount of 0.75% senior subordinated convertible notes due in October 2018 (the 2018 Notes) and $375.0 million principal amount of 1.50% senior subordinated convertible notes due in October 2020 (the 2020 Notes and, together with the 2018 Notes, the

Notes). Interest expense in the three and six months ended June 30, 2017, compared to the three and six months ended June 30, 2016 remained relatively flat. We do not expect interest expense to fluctuate significantly over the next 12 months. See Note 11 to our accompanying Condensed Consolidated Financial Statements for additional information regarding our debt.

Benefit from Income Taxes

For the three and six months ended June 30, 2017, we recognized a benefit from income taxes of $8.7 million and $16.7 million, respectively, compared to the three and six months ended June 30, 2016 when we recognized an income tax benefit of $163.9 million and $170.0 million, respectively. The benefit from income taxes for the three and six months ended June 30, 2016 primarily included the reversal of the deferred tax liability associated with the write-off of the IPR&D related to Kyndrisa and reveglucosidase alfa. We have historically computed our interim period 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 can be highly sensitive to minor fluctuations in U.S. forecasted income. As such, we have computed the U.S. component of the consolidated benefit from income taxes for the three and six months ended June 30, 2017 and 2016 using an actual year-to-date tax calculation. Foreign tax expense was computed using a forecasted annual effective tax rate for the three and six months ended June 30, 2017 and 2016.

The benefit from income taxes for the three and six months ended June 30, 2017 and 2016 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, which resulted in a net tax benefit in both periods. See Note 15 to our Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2016 for additional discussion of the components of our benefit from income taxes.

Financial Position, Liquidity and Capital Resources

As of June 30, 2017, we had $1.2 billion in cash, cash equivalents, and short-term and long-term investments. We expect to fund our operations with our net product revenues from our commercial products, cash, cash equivalents, and short-term and long-term investments, supplemented by proceeds from equity or debt financings and loans, or collaborative agreements with corporate partners. We may require additional financing to fund the repayment of our convertible debt, future milestone payments and our future operations, including the commercialization of our products and product candidates currently under development, preclinical studies and clinical trials, and potential licenses and acquisitions. 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 timing and mix of our funding options could change depending on many factors, including 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.

In managing our liquidity needs in the U.S., we do not rely on unrepatriated earnings as a source of funds and we have not provided for U.S. federal or state income taxes on these undistributed foreign earnings. As of June 30, 2017, $117.3 million of our $1.2 billion balance of cash, cash equivalents, and investments was held in foreign subsidiaries, a significant portion of which is required to fund the liquidity needs of these foreign subsidiaries. For additional discussion regarding income taxes, see Note 15 to our Consolidated Financial Statements included in our Annual Report on Form 10-K for the year ended December 31, 2016.

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 June 30, 2017 and December 31, 2016 were as follows (in millions):

| | June 30, 2017 | December 31, 2016 | Change |
|---|---|---|---|
| Cash and cash equivalents | $354.9 | $ 408.3 | $(53.4) |
| Short-term investments | 372.9 | 381.3 | (8.4) |
| Long-term investments | 482.0 | 572.8 | (90.8) |
| Cash, cash equivalents and investments | $1,209.8 | $ 1,362.4 | $(152.6) |
| | | | |
| Convertible debt | $676.2 | $ 683.2 | $(7.0) |

Our cash flows for the six months ended June 30, 2017 and 2016 are summarized as follows (in millions):

| | 2017 | 2016 | Change |
|---|---|---|---|
| Cash and cash equivalents at the beginning of the period | $408.3 | $397.0 | $11.3 |
| Net cash used in operating activities | (61.7) | (217.3) | 155.6 |
| Net cash (used in) provided by investing activities | (14.8) | 152.5 | (167.3) |
| Net cash provided by (used in) financing activities | 12.1 | (29.8) | 41.9 |
| Foreign exchange impact | 11.0 | 3.6 | 7.4 |
| Cash and cash equivalents at the end of the period | $354.9 | $306.0 | $48.9 |
| Short-term and long-term investments | 854.9 | 398.9 | 456.0 |
| Cash, cash equivalents and investments | $1,209.8 | $704.9 | $504.9 |

Cash Used in Operating Activities

Cash used in operating activities for the six months ended June 30, 2017 was $61.7 million, compared to cash used in operating activities of $217.3 million for the six months ended June 30, 2016. Cash used in operating activities primarily consisted of net loss of $53.1 million, adjusted for non-cash items such as $70.8 million for stock-based compensation expenses, $38.5 million for depreciation and amortization expense, $15.6 million of non-cash interest expense, partially offset by $22.8 million for deferred income taxes and $3.3 million gain on the sale of strategic investments. Changes in operating assets and liabilities resulted in a net cash outflow of $114.8 million that consisted primarily of increased cash outflow for increased inventory spending for all commercial products to meet anticipated future sales demand and payments for R&D purchases.

Cash Used in (Provided by) Investing Activities

Net cash used in investing activities during the six months ended June 30, 2017 was $14.8 million, compared to net cash provided by investing activities during the six months ended June 30, 2016 of $152.5 million. The increase in net cash used in investing activities for the six months ended June 30, 2017 compared to the six months ended June 30, 2016 primarily consisted of a $121.2 million increase in net purchases of available-for-sale securities and a $46.1 million increase of capital purchases. We expect to continue to make significant capital investments in our manufacturing and administrative facilities to accommodate anticipated headcount growth.

Cash Provided by (Used in) Financing Activities

Net cash provided by financing activities for the six months ended June 30, 2017 was $12.1 million, compared to net cash used in financing activities of $29.8 million for the six months ended June 30, 2016. The increase in net cash provided by financing activities for the six months ended June 30, 2017 was primarily attributable to a $17.6 million increase in proceeds from employee stock option exercises and purchases pursuant to the Employee Stock Purchase Plan, and a $26.2 million decrease in taxes paid related to net share settlement of employee equity awards.

Other Information

The 2017 Notes matured on April 23, 2017, with conversion of all principal amounts except for a final cash settlement of $26,000. Our indebtedness consists primarily of the 2018 and 2020 Notes, which, if not converted, will be required to be repaid in cash at maturity in 2018 and 2020, respectively. Our $750.0 million (undiscounted) of total convertible debt as of June 30, 2017 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. 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. Our liquidity could be adversely affected if we are required to settle the principal amount of our conversion obligation in cash. 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 (for example, if there are 12 months or less remaining until maturity), 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. We may seek to refinance or repay these obligations through funds raised from third-party financing, or equity or debt financings, none of which may be available on commercially reasonable terms, if at all. In addition, 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.

On August 12, 2016, we sold 7.5 million shares of our common stock at a price of $96.00 per share in an underwritten public offering pursuant to an effective registration statement previously filed with the SEC. We received net proceeds

of approximately $712.9 million from this public offering after accounting for the underwriting discount and offering costs.

In November 2016 we entered into a new Credit Agreement providing for up to $100.0 million in revolving loans (the Revolving Credit Facility). We expect to use the proceeds of the Revolving Credit Facility to finance ongoing working capital needs (including timing differences resulting from the strategic reduction of short-term investments) and for other general corporate purposes. As of June 30, 2017, we had not drawn on the Revolving Credit Facility. Although quarterly interest payments will be due on any outstanding balance due, we anticipate any balance due to be short-term in nature. See Note 11 to our accompanying Condensed Consolidated Financial Statements for additional discussion.

We sell our products in other countries, including Southern European countries, Russia, Chile and Brazil, which 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 customers in those countries to be unable to pay for our products.

Funding Commitments

We cannot estimate with certainty the cost to complete any of our product development programs. Additionally, 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 product candidates, or if approval of our product candidates is delayed, we will be unable to generate revenue from the sale of these product candidates, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will increase;
- If we are unable to successfully develop and maintain manufacturing processes for our 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 product candidates may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

Our investment in our product development programs and continued development of our existing commercial products has a major impact on our operating performance. Our R&D expenses of our major development programs from inception to June 30, 2017 were as follows (in millions):

| | Since Program Inception |
|---|---|
| BMN 250 | $ 119.5 |
| BMN 270 | 175.0 |
| Brineura | 216.9 |
| Pegvaliase | 457.2 |
| Vosoritide | 200.5 |
| Other approved products | 945.0 |
| 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 our commercial products; pre-clinical 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 our products;
- manufacturing, supply or distribution of our product candidates and commercial products;
- 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 currently pending lawsuit against DRL to protect our patents relating to Kuvan powder and generic competition to Kuvan relating to our settlements with DRL (related to Kuvan tablets) and Par (related to Kuvan tablets and powder);
- 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;
- negative publicity about our company or the pharmaceutical industry;
- broad market fluctuations in the U.S., the EU or in other parts of the world;
- actual or anticipated fluctuations in our operating results, including due to timing of large order for our products, in particular in Latin America, where governments place large periodic orders for Vimizim and Naglazyme;
- changes in company assessments or financial estimates by securities analysts;
- acquisitions of products, businesses, or other assets; 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.

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 certain development and regulatory activities and commercial sales and licensing milestones totaling approximately $585.5 million as of June 30, 2017, which are due upon achievement of certain development and commercial milestones, and if they occur before certain dates in the future. Of this amount, $210.9 million (USD equivalent of 185 million Euros translated at 1.14 USD per Euro) relates to the Merck PKU Business acquisition and $52.2 million relates to programs that are no longer being developed. See Note 18 to our accompanying Condensed Consolidated Financial Statements for additional discussion.

Other than as set forth above, there have been no material changes to our contractual and commercial obligations during the six months ended June 30, 2017, as compared to the significant accounting policies disclosed in Management’s Discussion and Analysis in our Annual Report on Form 10-K for the year ended December 31, 2016.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Our market risks during the six months ended June 30, 2017 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, 2016, which was filed with the SEC on February 27, 2017.

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 as of June 30, 2017.

In designing and evaluating our disclosure controls and procedures, our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management must apply its judgment in evaluating the cost-benefit relationship of possible

controls and procedures. Accordingly, our disclosure controls and procedures are designed to provide reasonable, not absolute, assurance that the objectives of our disclosure controls system are met.

(b) Change in Internal Control 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 October 3, 2014, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, 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 FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Together with Merck & Cie, on November 17, 2014, we filed a lawsuit against DRL 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 DRL from introducing a generic version of Kuvan tablets that would infringe our patents prior to their expiration. In September 2015, we and Merck & Cie entered into a settlement agreement with DRL (the DRL Tablet Settlement Agreement) that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral tablets. Under the terms of the DRL Tablet Settlement Agreement, we granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg oral tablets in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances.

Additionally, we received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (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 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 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.

We also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying us that Par had 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.

In April 2017, we and Merck & Cie entered into a settlement agreement with Par (the Par Settlement Agreement) that resolved both cases against Par. Under the Par Settlement Agreement, we granted Par a non-exclusive license to our Kuvan-related patents to allow Par to market a generic version of sapropterin dihydrochloride in 100 mg oral tablets and oral powder in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan

beginning on: April 1, 2021 if Par is not entitled to the statutory 180-day first filer exclusivity period; October 1, 2020 if Par is entitled to the statutory 180-day first filer exclusivity period; or earlier under certain circumstances.

We also received a paragraph IV notice letter, dated December 23, 2016, from DRL, notifying us that DRL had 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 6, 2017, we filed a lawsuit against DRL 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 DRL 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 DRL’s ANDA in accordance with the Hatch-Waxman Act, which expires in June 2019 or earlier if the court enters judgment finding that each of the then-asserted patents is invalid or not infringed, or such shorter or longer period as the court may order. DRL filed its answer to the complaint on April 10, 2017, alleging, inter alia, that the patents are not infringed and/or are invalid.  Neither the DRL Tablet Settlement Agreement nor the Par Settlement Agreement affects this currently pending litigation against DRL relating to Kuvan 100 mg oral powder.

SEC Subpoena

In August 2016, we received a subpoena from the staff of the Securities and Exchange Commission (SEC) requesting that we produce documents in connection with a non-public, fact-finding inquiry related to our former drisapersen program. The letter enclosing the subpoena states that the investigation and the subpoena do not mean that the Company or anyone else has broken the law, or that the SEC has a negative opinion of any person, entity or security. We intend to cooperate fully with the SEC in this matter. We are not able to predict whether any proceeding may be instituted in connection with the subpoena, or the outcome of any proceeding that may be instituted.

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, 2016, which was filed with the SEC on February 27, 2017.

Risks Related to Our Business

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

We must obtain and maintain regulatory approval to market and sell our product candidates. For example, in the U.S., we must obtain Food and Drug Administration (FDA) approval for each product candidate that we intend to commercialize, and in Europe we must obtain approval from the European Medicines Agency (EMA). The FDA and EMA approval processes are typically lengthy and expensive, and approval is never certain. Accordingly, there are no assurances that we will obtain regulatory approval for any of our product candidates, including pegvaliase, in any jurisdiction. For example, even though the pivotal Phase 3 PRISM-2 study of pegvaliase met the primary endpoint of change in blood Phe compared with placebo ($p<0.0001$), we did not demonstrate a statistically significant improvement in inattention or mood scores, a key secondary clinical neurocognitive endpoint. We filed a Biologics License Application (BLA) for pegvaliase with the FDA in the second quarter of 2017, but there is no assurance that a reduction in blood Phe alone will be sufficient to support the FDA’s full regulatory approval of pegvaliase.

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 product candidates. 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 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 separately regulated 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 product candidates, we engage in discussions with the FDA and comparable international regulatory authorities regarding our development programs, including discussions about the regulatory requirements for approval. As part of these discussions, we sometimes seek advice in the design of our clinical programs from various regulatory agencies globally, but we do not always follow such guidance. This increases the chance of adverse regulatory actions, but we try to always provide appropriate scientific evidence to support approval. Also, sometimes different regulatory agencies provide different or conflicting advice. While we attempt to harmonize the advice we receive from multiple regulatory authorities, it is not always practical to do so. Also, we may choose not to harmonize conflicting advice when harmonization would significantly delay clinical trial data or is otherwise inappropriate. 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 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.

All of our products have received regulatory approval to be commercially marketed and sold in the U.S., the EU and certain other countries, with the exception of Firdapse, which 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.

In addition, the FDA often requires post-marketing testing and surveillance to monitor the effects of 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 of our 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 products is limited, orphan drug designation is especially important for our products that are eligible for orphan drug designation. For eligible products, 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 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 biosimilars approved through an abbreviated regulatory pathway.

Our Aldurazyme, Brineura, Naglazyme 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 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 exclusivity for reference products. Aldurazyme's exclusivity under the BPCIA expired in 2015, Brineura's exclusivity under the BPCIA expires in 2029, Naglazyme's exclusivity under the BPCIA expired June 1, 2017, and Vimizim's 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 drug development process we must conduct, at our own expense, preclinical studies in the laboratory, including studies in animals, 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, new drugs for diseases or conditions that affect larger patient populations, are less severe, or are treatable by alternative strategies must be validated through additional preclinical and clinical trials and/or clinical trials with higher enrollments. With respect to our early stage product candidates, we may need to perform multiple preclinical studies using various doses and formulations before we can begin clinical trials, which could result in delays to our development timeline. 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 product candidates 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, including us with respect to Kyndrisa, have suffered significant setbacks in advanced clinical trials due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier trials. Also, as noted above, we do not always follow the advice of regulatory authorities or comply with all of their requests regarding the design of our clinical programs. In those cases, we may choose a development program that is inconsistent with the advice of regulatory authorities, which may limit the jurisdictions where we conduct clinical trials and/or adversely affect our ability to obtain approval in those jurisdictions where we do not follow the regulatory advice.

Adverse or inconclusive clinical results could stop us from obtaining 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;
- budgetary constraints or prohibitively high clinical trial costs;
- longer treatment time required to demonstrate efficacy;
- lack of sufficient supplies of the product candidate;
- adverse medical events or side effects in treated patients, including immune reactions;
- lack of effectiveness of the product candidate being tested;
    - availability of competitive therapies to treat the same indication as our product candidates;
- regulatory requests for additional clinical trials or pre-clinical studies;
- deviations in standards for Good Clinical Practice (GCP); and

- disputes with or disruptions in our relationships with clinical trial partners, including CROs, clinical laboratories, clinical sites, and principal investigators

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services reportable to the FDA or other regulatory authority. If the FDA or other regulatory authority concludes that a financial relationship between us and a principal investigator has created a conflict of interest, the FDA or other regulatory authority may question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized.

Our BMN 270 program is based on a gene therapy approach, which, as a novel technology, presents additional treatment, regulatory, manufacturing, and commercial risks in relation to our other, more traditional drug development programs.

In addition to the risks set forth in this Risk Factors section associated with developing and commercializing more traditional pharmaceutical drugs, there are additional, unique risks associated with gene therapy products like our product candidate BMN 270. The goal of gene therapy is to be able to correct an inborn genetic defect through one-time administration of therapeutic genetic material containing non-defective gene copies. The gene copies are designed to reside permanently in a patient, allowing the patient to produce an essential protein or ribonucleic acid (RNA) molecule that a healthy person would normally produce. There is a risk, however, that the new gene copies will produce too much or too little of the desired protein or RNA. There is also a risk that production of the desired protein or RNA will increase or decrease over time. Because the treatment is irreversible, there may be challenges in managing side effects, particularly those caused by overproduction. Adverse effects would not be able to be reversed or relieved by stopping dosing, and we may have to develop additional clinical safety procedures. Furthermore, because the new gene copies are designed to reside permanently in a patient, there is a risk that they will disrupt other normal biological molecules and processes, including other healthy genes, and we may not learn the nature and magnitude of these side effects until long after clinical trials have been completed.

We may experience development problems related to our gene therapy program that cause significant delays or unanticipated costs, or that cannot be solved. Given that there are currently no approved gene therapy products in the U.S. and very few precedents outside the U.S., it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidate in any jurisdiction. Regulatory requirements governing gene and cell therapy products are still evolving and may continue to change in the future. Regulatory review agencies and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our treatment candidate or lead to significant post-approval studies, limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring BMN 270 to market could have a negative effect on our business and financial condition. Even if we do obtain regulatory approval, ethical, social and legal concerns about gene therapy arising in the future could result in additional regulations restricting or prohibiting sale of our product.

Even if we obtain regulatory approval for BMN 270, we may experience delays, and increased costs, in developing a sustainable, reproducible and large-scale manufacturing process. Gene therapy products are novel, complex and difficult to manufacture, and have, only in limited cases, been manufactured at scales sufficient for pivotal trials and commercialization. Few pharmaceutical contract manufacturers specialize in gene therapy products and those that do are still developing appropriate processes and facilities for large-scale production. Whether we produce BMN 270 at a contract manufacturer or at our own gene therapy manufacturing facility, we will likely face technical and scientific challenges, considerable capital costs, and potential difficulty in recruiting and hiring experienced, qualified personnel. As a result, we could experience manufacturing delays that prevent us from completing our clinical studies or commercializing BMN 270 in a timely, or on a profitable, basis, if at all.

Due to the relative novelty of gene therapy and the potential to provide extended duration therapeutic treatment with a one-time administration, we also face uncertainty with respect to the pricing, coverage and reimbursement of BMN 270, if approved. In order to recover our research and development costs and commercialize this one-time treatment on a profitable basis, we expect the cost of a single administration of BMN 270 to be substantial. Therefore, we expect that coverage and reimbursement by governments and other third party payors will be essential for the vast majority of patients to be able to afford BMN 270. Accordingly, sales of BMN 270, if approved, will depend substantially, both domestically and internationally, on the extent to which its cost will be paid by third party payors. Even if coverage is provided, the reimbursement amounts approved by third party payors may not be high enough to allow us to realize a

sufficient return on our investment.

We also face uncertainty as to whether gene therapy will gain the acceptance of the public or the medical community. Even if we obtain regulatory approval for BMN 270, the commercial success of BMN 270 will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidate in particular, as medically necessary, cost-effective and safe. In particular, our success will depend upon physicians prescribing our product candidate in lieu of existing treatments they are already familiar with and for which greater clinical data may be available. Even if BMN 270 displays a favorable efficacy and safety profile in clinical trials and is ultimately approved, market acceptance of BMN 270 will not be fully known until after it is launched. Negative public opinion or more restrictive government regulations or could have a negative effect on our business and financial condition and may delay or impair the development and commercialization of, and demand for, BMN 270.

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 needs, such as for 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 our products, the receipt of regulatory approval of our product candidates, our ability to successfully manufacture and market any products, 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 June 30, 2017, we had cash, cash equivalents and short and long-term investments totaling $1.2 billion and long-term debt obligations of $750.0 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 $374.5 million in the six months ended June 30, 2017, 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 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 may require additional financing to fund the repayment of our Notes, future milestone payments and our future operations, including the commercialization of our products and 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 our products;
    - 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., 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.

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 June 30, 2017, we had $750.0 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. In November 2016, we also entered into a credit agreement (Credit Agreement) with Bank of America, N.A., as the administrative agent, swing line lender and letter of credit issuer, providing for up to $100.0 million in revolving loans. 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;
- 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, if we default under the Credit Agreement, the outstanding borrowings thereunder could become immediately due and payable, the Credit Agreement lenders could refuse to permit additional borrowings under the facility, or it could lead to defaults under agreements governing our current or future indebtedness, including the indentures governing our 2018 Notes and 2020 Notes.

*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 the 2018 and 2020 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. Our liquidity could be adversely affected if we are required to settle the principal amount of our conversion obligation in cash. 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 (for example, if there are 12 months or less remaining until maturity), 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.

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*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 scheduled and unannounced inspection by the FDA and international regulatory authorities, before and after product approval, to monitor and ensure compliance with cGMP and other regulations. Our manufacturing facility in the U.S. has been approved by the FDA, the EC, and health agencies in other countries for the manufacture of Aldurazyme, Brineura, 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 our products 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 our products 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 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 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 (including moving manufacturing from one of our facilities to another one of our facilities or a third party facility, or from a third party facility to one of our facilities) may require us to complete clinical trials to receive regulatory approval of any manufacturing modifications.

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 Aldurazyme, Brineura, Naglazyme 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 Firdapse and Kuvan, if those manufacturers are unwilling or unable to fulfill their contractual obligations, we may be unable to meet demand for Firdapse and Kuvan 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 Firdapse and Kuvan. We also rely on third parties for portions of the manufacture of Aldurazyme, Brineura, Naglazyme 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 incur significant costs in complying with these laws and regulations.

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 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 Brineura, 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, 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 products by third party payors, the sales of our products 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 payors. 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 payors, 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 payor, 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 payors 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 payors 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 payors in the U.S. Therefore, coverage and reimbursement for drug products can differ significantly from payor to payor. 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 payor 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 will exceed 12 months. Even after a price is negotiated, countries frequently request or require reductions 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, and we expect a significant portion of our international sales of Brineura will also be through such programs. 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 in certain jurisdictions. 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, which may prevent us from marketing our product entirely) 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 payors 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 payors, 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 our products. 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 product 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 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 U.S. the Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act of 2010 (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.

Since its enactment, 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. In January 2017, President Trump signed an Executive Order directing federal agencies with authorities and responsibilities under the PPACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the PPACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. In addition, the new Presidential Administration and U.S. Congress have attempted and will likely continue to seek to modify, repeal, or otherwise invalidate all, or certain provisions of, the PPACA. It is uncertain the extent to which any such changes may impact our business or financial condition.

In addition, other legislative changes have been adopted since the PPACA was enacted. Some of these changes have resulted 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. Recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products. For example, 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, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drug products. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. In addition, individual states in the United States have also become increasingly active in passing legislation and implementing regulations designed to control pharmaceutical product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. Legally mandated price controls on payment amounts by third-party payors or other restrictions could harm our business, results of operations, financial condition and prospects. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. 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. For more information regarding government health care reform, see “Government Regulation - Health Reform” in Part I, Item 1 of our Annual Report on Form 10-K for the year ended December 31, 2016, filed with the SEC on February 27, 2017.

We face credit risks from government-owned or sponsored 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 payors.

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, or knowingly making, using, or causing to be made or used, a false record or statement to avoid, decrease or conceal an obligation to pay money to the federal government. 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 payors, 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. For example, in August 2016, we received a subpoena from the staff of the SEC requesting that we produce documents in connection with a non-public, fact-finding inquiry related to our former drisapersen program. The letter enclosing the subpoena states that the investigation and the subpoena do not mean that the Company or anyone else has broken the law, or that the SEC has a negative opinion of any person, entity or security. We intend to cooperate fully with the SEC in this matter. We are not able to predict whether any proceeding may be instituted in connection with the subpoena, or the outcome of any proceeding that may be instituted.

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, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, 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, Kuvan, Naglazyme and Vimizim, and all of the sales of Firdapse are generated from countries other than the U.S. Similarly, we expect a significant portion of the sales of Brineura to be 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, exposure to fluctuations in foreign currency exchange rates and potential currency controls imposed by foreign governments;
- 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.

*If we fail to comply with U.S. export control and economic sanctions, our business, financial condition and operating results may be adversely affected.

We rely on a general license from the U.S. Treasury Department's Office of Foreign Assets Control (OFAC) to sell our medicines for eventual use by hospital and clinic end-users in Iran. The use of this OFAC general license requires us to observe strict conditions with respect to products sold, end-user limitations and payment requirements. Although we believe we have maintained compliance with the general license requirements, there can be no assurance that the general license will not be revoked, be renewed in the future or that we will remain in compliance. Moreover, a violation of the OFAC general license could result in substantial fines, sanctions, civil or criminal penalties, competitive or reputational harm, litigation or regulatory action and other consequences that might adversely affect our results of operations, financial condition or strategic objectives.

*Failure to comply with applicable anti-corruption legislation could result in fines, criminal penalties and materially adversely affect our business, financial condition and results of operations.

We are required to comply with anti-corruption and anti-bribery laws in the jurisdictions in which we operate, including the FCPA in the United States, the UK Bribery Act and other similar laws in other countries in which we do business. We operate in a number of countries that are recognized to have a reputation for corruption and pose an increased risk of corrupt practices. We also regularly interact with government regulators in many countries, including those that are considered higher risk for corruption, in order to secure regulatory approval to manufacture and distribute our products. The anti-corruption and anti-bribery laws to which we are subject generally prohibit companies and their intermediaries from making improper payments to foreign officials or other persons for the purposes of influencing official decisions or obtaining or retaining business and/or other benefits. These laws also require us to make and keep books and records that accurately and fairly reflect our transactions and to devise and maintain an adequate system of internal accounting controls. As part of our business, we deal with state-owned business enterprises, the employees and representatives of which may be considered foreign officials for purposes of applicable anti-corruption laws.

Although we have adopted policies and procedures designed to ensure that the Company, our employees and third party agents will comply with such laws, there can be no assurance that such policies or procedures will work effectively at all times or protect us against liability under these or other laws for actions taken by our employees, partners and other third parties with respect to our business. If we are not in compliance with anti-corruption laws and other laws governing the conduct of business with government entities and/or officials (including local laws), we may

be subject to criminal and civil penalties and other remedial measures, which could harm our business, financial condition, results of operations, cash flows and prospects. Investigations of any actual or alleged violations of such laws or policies related to us could harm our business, financial condition, results of operations, cash flows and prospects.

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.

We 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 Aldurazyme, Naglazyme 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 our products. 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 notwithstanding our filed patents or patent applications.
- Enforcing patents is expensive and may absorb significant time of our management. Management would spend less time and resources on developing products, which could increase our operating expenses and delay product programs.
- 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.

Under policies recently adopted in the EU, clinical trial data submitted to the EMA in MAAs that were traditionally regarded as confidential commercial information are now subject to public disclosure. Subject to BioMarin's ability to review and redact a narrow sub-set of confidential commercial information, the new EU policies will result in the EMA's public disclosure of certain of our clinical study reports, clinical trial data summaries and clinical overviews for recently completed and future MAA submissions. 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 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 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.

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 and several of our former and current product programs have been developed through licensing or collaborative arrangements, such as Aldurazyme, Firdapse, Kuvan and Naglazyme. 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 are successful in their use of litigation or 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 Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). The Hatch-Waxman Act requires an ANDA applicant seeking FDA approval of its proposed generic product prior to the expiration of our Orange Book-listed patents to certify that the applicant believes that our patents are invalid or will not be infringed by the manufacture, use or sale of the drug for which the application has been submitted (a paragraph IV certification) and notify us of such certification (a paragraph IV notice). Upon receipt of a paragraph IV notice, the Hatch-Waxman Act allows us, with proper basis, to bring an action for patent infringement against the ANDA filer, asking that the proposed generic product not be approved until after our patents expire. If we commence a lawsuit within 45 days from receipt of the paragraph IV notice, the Hatch-Waxman Act provides a 30-month stay, during which time the FDA cannot finally approve the generic's application. If the litigation is resolved in favor of the ANDA applicant during the 30-month stay period, the stay is lifted and the FDA 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 Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, 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 FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). Together with Merck & Cie, on November 17, 2014, we filed a lawsuit against DRL 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 DRL from introducing a generic version of Kuvan tablets that would infringe our patents prior to their expiration. In September 2015, we and Merck & Cie entered into a settlement agreement with DRL (the DRL Tablet Settlement Agreement) that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral tablets. Under the terms of the DRL Tablet Settlement Agreement, we granted DRL a non-exclusive license to our Kuvan-related patents to allow DRL to market a generic version of sapropterin dihydrochloride 100 mg oral tablets in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances.

Additionally, we received a paragraph IV notice letter, dated January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying us that Par had filed an ANDA seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral tablets prior to the expiration of our patents listed in the FDA's Orange Book. Together with Merck & Cie, on March 6, 2015, 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 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.

We also received a paragraph IV notice letter, dated January 14, 2016, from Par, notifying us that Par had 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.

In April 2017, we and Merck & Cie entered into a settlement agreement with Par (the Par Settlement Agreement) that resolved both cases against Par. Under the Par Settlement Agreement, we granted Par a non-exclusive license to our Kuvan-related patents to allow Par to market a generic version of sapropterin dihydrochloride in 100 mg oral tablets and oral powder in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on: April 1, 2021 if Par is not entitled to the statutory 180-day first filer exclusivity period; October 1, 2020 if Par is entitled to the statutory 180-day first filer exclusivity period; or earlier under certain circumstances.

We also received a paragraph IV notice letter, dated December 23, 2016, from DRL, notifying us that DRL had 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 6, 2017, we filed a lawsuit against DRL 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 DRL 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 DRL’s ANDA in accordance with the Hatch-Waxman Act, which expires in June 2019 or earlier if the court enters judgment finding that each of the then-asserted patents is invalid or not infringed, or such shorter or longer period as the court may order. DRL filed its answer to the complaint on April 10, 2017, alleging, inter alia, that the patents are not infringed and/or are invalid. Neither the DRL Tablet Settlement Agreement nor the Par Settlement Agreement affects this currently pending litigation against DRL relating to Kuvan 100 mg oral powder. That litigation matter is still pending. For more information regarding these matters, see “Legal Proceedings” in Part II, Item 1 of this Interim Report on Form 10-Q.

The DRL Tablet Settlement Agreement, the Par Settlement Agreement, and the filing of DRL’s purported ANDA with respect to Kuvan powder, as well as any future ANDA or related legal proceeding, 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 DRL 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 (relating to Kuvan tablets) and Par (relating to Kuvan tablets and powder) following the settlements described above could have a material adverse effect on our revenue and results of operations.

We also face potential generic competition for Kuvan in certain foreign countries, and our ability to successfully market and sell Kuvan in many countries in which we operate is based upon patent rights or certain regulatory forms of exclusivity, or both. The scope of our patent rights and regulatory exclusivity for Kuvan vary from country to country and are dependent on the availability of meaningful legal remedies in each country. If our patent rights and regulatory exclusivity for Kuvan are successfully challenged, expire, or otherwise terminate in a particular country, the resulting generic competition 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 license or acquire in the future may be intended for patient populations that are significantly larger than any of the patient populations we currently target. 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 product candidates are approved, if doctors elect a course of treatment which does not include our products, this decision would reduce demand for our 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 Aldurazyme, Naglazyme, and Vimizim in MPS diseases, could be greatly reduced. Moreover, if we obtain regulatory approval for BMN 270, the commercial success of BMN 270 will still depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidate in particular, as medically necessary, cost-effective and safe. 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.

*If a natural disaster or terrorist or criminal activity caused significant damage to our facilities or the facilities of our third party manufacturers and suppliers, 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 Aldurazyme, Brineura, Naglazyme 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 Aldurazyme, Brineura, Naglazyme and Vimizim or our third party manufacturers' ability to manufacture Firdapse or Kuvan.

Our Galli Drive facility located in Novato, California is currently our only manufacturing facility for Aldurazyme and Naglazyme and is one of two manufacturing facilities for Brineura and Vimizim. It is located in the San Francisco Bay Area near known earthquake fault zones and is vulnerable to significant damage from earthquakes. We, the third party manufacturers with whom we contract and our single-source suppliers of raw materials, which include many of our critical raw materials, are also vulnerable to damage from other types of disasters, including fires, explosions, 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 Aldurazyme, Brineura, Naglazyme and Vimizim, or to have Firdapse or Kuvan 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.

*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.

We sell our products in countries that face economic crises and local currency devaluation, including Southern European countries, Russia, Chile and Brazil. Although we have 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 our products. Additionally, if one or more of these countries were unable to purchase our products, our revenue would be adversely affected.

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.

Risks Related to Ownership of Our Securities

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

Our valuation and stock price 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 our products;
- manufacturing, supply or distribution of our product candidates and commercial products;
- 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 currently pending lawsuit against DRL to protect our patents relating to Kuvan powder and generic competition to Kuvan relating to our settlements with DRL (related to Kuvan tablets) and Par (related to Kuvan tablets and powder);
- government regulatory action affecting our product candidates, our products or our competitors' product candidates and 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;
- negative publicity about our company or the pharmaceutical industry;
- broad market fluctuations in the U.S., the EU or in other parts of the world;
- actual or anticipated fluctuations in our operating results, including due to timing of large order for our products, in particular in Latin America, where governments place large periodic orders for Naglazyme and Vimizim;
- changes in company assessments or financial estimates by securities analysts;
- acquisitions of products, businesses, or other assets; and
- sales of our shares of stock by us, our significant stockholders, 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 restated certificate of incorporation and amended and restated bylaws providing that stockholders' meetings may only be called by our Chairman, the lead independent director or the majority of our Board of Directors and 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
Not applicable.

Item 5. Other Information.
None.

Item 6. Exhibits.

| Exhibit Number | Description |
|---|---|
| 2.1 | Purchase Agreement, dated as of November 23, 2014, among BioMarin Falcons B.V., BioMarin Pharmaceutical Inc. and Prosensa Holding N.V., previously filed with the SEC on November 26, 2014 as Exhibit 2.01 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated by reference herein. |
| 2.2 | Amended and Restated Termination and Transition Agreement, dated as of December 23, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |
| 2.3 | Termination Agreement, dated as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |
| 2.4 | Termination and Transition Agreement, dated as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |
| 2.5 | First Amendment, dated as of December, 12, 2016, to the Amended and Restated Termination and Transition Agreement, dated as of December 23, 2015 and effective as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on February 27, 2017 as Exhibit 2.6 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |
| 3.1 | Restated Certificate of Incorporation of BioMarin Pharmaceutical Inc., previously filed with the SEC on June 12, 2017 as Exhibit 3.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. |
| 3.2 | Amended and Restated Bylaws of BioMarin Pharmaceutical Inc., previously filed with the SEC on June 15, 2015 as Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. |
| 10.1* | Settlement and License Agreement among BioMarin Pharmaceutical Inc., Merck & Cie and Par Pharmaceutical, Inc., dated as of April 12, 2017. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |

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| 10.2 | BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. |
| 10.3 | Form of Stock Options Agreement for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. |
| 10.4 | Form of Agreement Regarding Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. |
| 10.5 | Form of Agreement Regarding Performance Compensation Award in the Form of Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.4 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. |
| 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. |

| Exhibit Number | Description |
|---|---|
| 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

+The certifications attached as Exhibit 32.1 accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, and shall not be deemed “filed” by the Registrant for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any of the Registrant’s filings under the Securities Act of 1933, as amended, irrespective of any general incorporation language contained in any such filing.

Attached as Exhibit 101 to this report are documents formatted in XBRL (Extensible Business Reporting Language): (i) Condensed Consolidated Balance Sheets as of June 30, 2017 and December 31, 2016, (ii) Condensed Consolidated Statements of Comprehensive Loss for the three and six months ended June 30, 2017 and 2016, (iii) Condensed Consolidated Statement of Stockholders’ Equity for the six months ended June 30, 2017, (iv) Condensed Consolidated Statements of Cash Flows for the six months ended June 30, 2017 and 2016, and (v) 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: August 2, 2017 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

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| 2.3 | Termination Agreement, dated as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |
| 2.4 | Termination and Transition Agreement, dated as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on January 7, 2016 as Exhibit 2.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |
| 2.5 | First Amendment, dated as of December, 12, 2016, to the Amended and Restated Termination and Transition Agreement, dated as of December 23, 2015 and effective as of October 1, 2015, between BioMarin Pharmaceutical Inc. and Ares Trading S.A., previously filed with the SEC on February 27, 2017 as Exhibit 2.6 to the Company's Annual Report on Form 10-K (File No. 000-26727), which is incorporated herein by reference. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with the SEC. |
| 3.1 | Restated Certificate of Incorporation of BioMarin Pharmaceutical Inc., previously filed with the SEC on June 12, 2017 as Exhibit 3.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. |
| 3.2 | Amended and Restated Bylaws of BioMarin Pharmaceutical Inc., previously filed with the SEC on June 15, 2015 as Exhibit 3.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference. |
| 10.1* | Settlement and License Agreement among BioMarin Pharmaceutical Inc., Merck & Cie and Par Pharmaceutical, Inc., dated as of April 12, 2017. Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. Omitted portions have been filed separately with |

the SEC.

10.2 BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.1 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.

10.3 Form of Stock Options Agreement for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.2 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.

10.4 Form of Agreement Regarding Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.3 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.

10.5 Form of Agreement Regarding Performance Compensation Award in the Form of Restricted Stock Units for the BioMarin Pharmaceutical Inc. 2017 Equity Incentive Plan, previously filed with the SEC on June 12, 2017 as Exhibit 10.4 to the Company's Current Report on Form 8-K (File No. 000-26727), which is incorporated herein by reference.

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.

| Exhibit Number | Description |
|---|---|
| 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

+The certifications attached as Exhibit 32.1 accompany this Quarterly Report on Form 10-Q pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, and shall not be deemed “filed” by the Registrant for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and are not to be incorporated by reference into any of the Registrant’s filings under the Securities Act of 1933, as amended, irrespective of any general incorporation language contained in any such filing.

Attached as Exhibit 101 to this report are documents formatted in XBRL (Extensible Business Reporting Language): (i) Condensed Consolidated Balance Sheets as of June 30, 2017 and December 31, 2016, (ii) Condensed Consolidated Statements of Comprehensive Loss for the three and six months ended June 30, 2017 and 2016, (iii) Condensed Consolidated Statement of Stockholders’ Equity for the six months ended June 30, 2017, (iv) Condensed Consolidated Statements of Cash Flows for the six months ended June 30, 2017 and 2016, and (v) Notes to Condensed Consolidated Financial Statements.