BIOMARIN PHARMACEUTICAL INC Form 10-Q October 31, 2017

#### UNITED STATES

#### SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-Q

(Mark One)

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

Or

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF1934For the transition period fromto

Commission File Number: 000-26727

BioMarin Pharmaceutical Inc.

(Exact name of registrant as specified in its charter)

Delaware68-0397820(State or other jurisdiction of(I.R.S. Employer)

incorporation or organization) Identification No.)

770 Lindaro Street, San Rafael, California94901(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: 175,621,277 shares of common stock, par value \$0.001, outstanding as of October 25, 2017.

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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<sup>®</sup>, Brineura<sup>®</sup>, Vimizim<sup>®</sup>, Naglazyme<sup>®</sup>, Kuvan<sup>®</sup> and Firdapse<sup>®</sup> are our registered trademarks. Kyndrisa<sup>TM</sup> is our trademark. Aldurazyme<sup>®</sup> 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," "n "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

#### September 30, 2017 and December 31, 2016

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

	September 30, 2017	December 31, 2016(1)
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	\$431,399	\$408,330
Short-term investments	825,700	381,347
Accounts receivable, net	251,891	215,280
Inventory	457,393	355,126
Other current assets	83,646	61,708
Total current assets	2,050,029	1,421,791
Noncurrent assets:		
Long-term investments	416,304	572,711
Property, plant and equipment, net	878,624	798,768
Intangible assets, net	530,957	553,780
Goodwill	197,039	197,039
Deferred tax assets	484,759	446,786
Other assets	22,985	32,815
Total assets	\$4,580,697	\$4,023,690
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	\$364,920	\$370,505
Short-term convertible debt, net		22,478
Short-term contingent acquisition consideration payable	52,609	46,327
Total current liabilities	417,529	439,310
Noncurrent liabilities:		
Long-term convertible debt, net	1,166,036	660,761
Long-term contingent acquisition consideration payable	126,790	115,310
Other long-term liabilities	56,780	42,034
Total liabilities	1,767,135	1,257,415
Stockholders' equity:		
Common stock, \$0.001 par value: 500,000,000 shares authorized; 175,495,350 and	176	173

172,647,588 shares issued and outstanding as of September 30, 2017 and December

31, 2016, respectively.					
Additional paid-in capital	4,435,449	4,288,113			
Company common stock held by Nonqualified Deferred Compensation Plan (NQDC)	(14,473	) (14,321 )			
Accumulated other comprehensive income (loss)	(21,434	) 12,816			
Accumulated deficit	(1,586,156	) (1,520,506)			
Total stockholders' equity	2,813,562	2,766,275			
Total liabilities and stockholders' equity	\$4,580,697	\$4,023,690			
(1) December 21, 2016 below and show derived from the endited Concellidated Einspecial Statements included in the					

(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 SEC on February 27, 2017.

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

## CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS

Three and Nine Months Ended September 30, 2017 and 2016

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

(Unaudited)

Other income, net       267       1,723       4,282       504         LOSS BEFORE INCOME TAXES       (20,621)       (66,813)       (90,473)       (738,884)         Benefit from income taxes       (8,094)       (29,388)       (24,823)       (199,394)         NET LOSS       \$(12,527)       \$(37,425)       \$(65,650)       \$(539,490)         NET LOSS PER SHARE, BASIC       \$(0.07)       \$(0.22)       \$(0.38)       \$(3.29)		Three Months Ended September 30,		Nine Mont September	
Net product revenues       \$298,752       \$278,262       \$916,868       \$812,195         Royalty and other revenues       35,396       1,634       38,473       4,568         Total revenues       334,148       279,896       955,341       816,763         OPERATING EXPENSES:              Cost of sales       59,480       50,738       165,791       145,473          Research and development       154,103       160,831       442,145       486,663          Selling, general and administrative       130,532       118,758       394,056       333,635         Intangible asset amortization and contingent consideration       3,760       9,654       26,096       (34,318)         Impairment of intangible assets       —       —       —       —       —       599,118         Total operating expenses       347,875       339,981       1,028,088       1,530,571         LOSS FROM OPERATIONS       (13,727)       (60,085)       (72,747)       (713,808)         Equity in the loss of BioMarin/Genzyme LLC       (253)       (104)       (996)       (374)       )         Interest expense       (10,884)       (9,980)       (31,043)       (29,767)		2017	2016	2017	2016
Royalty and other revenues35,3961,63438,4734,568Total revenues334,148279,896955,341816,763OPERATING EXPENSES: </td <td>REVENUES:</td> <td></td> <td></td> <td></td> <td></td>	REVENUES:				
Total revenues       334,148       279,896       955,341       816,763         OPERATING EXPENSES:       50,738       165,791       145,473         Research and development       154,103       160,831       442,145       486,663         Selling, general and administrative       130,532       118,758       394,056       333,635         Intangible asset amortization and contingent consideration       3,760       9,654       26,096       (34,318)         Impairment of intangible assets       -       -       -       599,118         Total operating expenses       347,875       339,981       1,028,088       1,530,571         LOSS FROM OPERATIONS       (13,727)       (60,085)       (72,747)       (713,808)         Equity in the loss of BioMarin/Genzyme LLC       (253)       (104)       (996)       (374)         Interest income       3,976       1,633       10,031       4,561         Interest expense       (10,884)       (9,980)       (31,043)       (29,767)         Other income, net       267       1,723       4,282       504         LOSS BEFORE INCOME TAXES       (20,621)       (66,813)       (90,473)       (738,884)         Benefit from income taxes       (8,094)       (29,388)	Net product revenues	\$298,752	\$278,262	\$916,868	\$812,195
OPERATING EXPENSES:         Cost of sales       59,480       50,738       165,791       145,473         Research and development       154,103       160,831       442,145       486,663         Selling, general and administrative       130,532       118,758       394,056       333,635         Intangible asset amortization and contingent consideration       3,760       9,654       26,096       (34,318)       )         Impairment of intangible assets       —       —       —       599,118       50,571       LOSS FROM OPERATIONS       (13,727)       (60,085)       (72,747)       (713,808)       )       Equity in the loss of BioMarin/Genzyme LLC       (253)       (104)       (996)       (374)       )         Interest income       3,976       1,633       10,031       4,561         Interest expense       (10,884)       (9,980)       (31,043)       (29,767)         Other income, net       267       1,723       4,282       504         LOSS BEFORE INCOME TAXES       (20,621)       (66,813)       (90,473)       (738,884)         Benefit from income taxes       (8,094)       (29,388)       (24,823)       (199,394)         NET LOSS       \$(12,527)       \$(37,425)       \$(65,650)       \$(539,490)	Royalty and other revenues	35,396	1,634	38,473	4,568
Cost of sales59,48050,738165,791145,473Research and development154,103160,831442,145486,663Selling, general and administrative130,532118,758394,056333,635Intangible asset amortization and contingent consideration3,7609,65426,096(34,318)Impairment of intangible assets———599,118Total operating expenses347,875339,9811,028,0881,530,571LOSS FROM OPERATIONS(13,727)(60,085)(72,747)(713,808)Equity in the loss of BioMarin/Genzyme LLC(253)(104)(996)(374)Interest income3,9761,63310,0314,561Interest expense(10,884)(9,980)(31,043)(29,767)Other income, net2671,7234,282504LOSS BEFORE INCOME TAXES(20,621)(66,813)(90,473)(738,884)Benefit from income taxes(8,094)(29,388)(24,823)(199,394)NET LOSS\$(12,527)\$(37,425)\$(65,650)\$(539,490)NET LOSS PER SHARE, BASIC\$(0.07)\$(0.22)\$(0.38)\$(3.29)	Total revenues	334,148	279,896	955,341	816,763
Research and development154,103160,831442,145486,663Selling, general and administrative130,532118,758394,056333,635Intangible asset amortization and contingent consideration3,7609,65426,096(34,318)Impairment of intangible assets599,118Total operating expenses347,875339,9811,028,0881,530,571LOSS FROM OPERATIONS(13,727)(60,085)(72,747)(713,808)Equity in the loss of BioMarin/Genzyme LLC(253)(104)(996)(374)Interest income3,9761,63310,0314,561Interest expense(10,884)(9,980)(31,043)(29,767)Other income, net2671,7234,282504LOSS BEFORE INCOME TAXES(20,621)(66,813)(90,473)(738,884)Benefit from income taxes(8,094)(29,388)(24,823)(199,394)NET LOSS\$(12,527)\$(37,425)\$(65,650)\$(539,490)NET LOSS PER SHARE, BASIC\$(0.07)\$(0.22)\$(0.38)\$(3.29)	OPERATING EXPENSES:				
Selling, general and administrative       130,532       118,758       394,056       333,635         Intangible asset amortization and contingent consideration       3,760       9,654       26,096       (34,318)         Impairment of intangible assets       -       -       -       599,118         Total operating expenses       347,875       339,981       1,028,088       1,530,571         LOSS FROM OPERATIONS       (13,727)       (60,085)       (72,747)       (713,808)         Equity in the loss of BioMarin/Genzyme LLC       (253)       (104)       (996)       (374)         Interest income       3,976       1,633       10,031       4,561         Interest expense       (10,884)       (9,980)       (31,043)       (29,767)         Other income, net       267       1,723       4,282       504         LOSS BEFORE INCOME TAXES       (20,621)       (66,813)       (90,473)       (738,884)         Benefit from income taxes       (8,094)       (29,388)       (24,823)       (199,394)         NET LOSS       \$(12,527)       \$(37,425)       \$(65,650)       \$(539,490)         NET LOSS PER SHARE, BASIC       \$(0.07)       \$(0.22)       \$(0.38)       \$(3.29)	Cost of sales	59,480	50,738	165,791	145,473
Intangible asset amortization and contingent consideration3,7609,65426,096(34,318)Impairment of intangible assets599,118Total operating expenses347,875339,9811,028,0881,530,571LOSS FROM OPERATIONS(13,727)(60,085)(72,747)(713,808)Equity in the loss of BioMarin/Genzyme LLC(253)(104)(996)(374)Interest income3,9761,63310,0314,561Interest expense(10,884)(9,980)(31,043)(29,767)Other income, net2671,7234,282504LOSS BEFORE INCOME TAXES(20,621)(66,813)(90,473)(738,884)Benefit from income taxes(8,094)(29,388)(24,823)(199,394)NET LOSS\$(12,527)\$(37,425)\$(65,650)\$(539,490)NET LOSS PER SHARE, BASIC\$(0.07)\$(0.22)\$(0.38)\$(3.29)	Research and development	154,103	160,831	442,145	486,663
Impairment of intangible assets       —       —       —       599,118         Total operating expenses       347,875       339,981       1,028,088       1,530,571         LOSS FROM OPERATIONS       (13,727)       (60,085)       (72,747)       (713,808)         Equity in the loss of BioMarin/Genzyme LLC       (253)       (104)       (996)       (374)         Interest income       3,976       1,633       10,031       4,561         Interest expense       (10,884)       (9,980)       (31,043)       (29,767)         Other income, net       267       1,723       4,282       504         LOSS BEFORE INCOME TAXES       (20,621)       (66,813)       (90,473)       (738,884)         Benefit from income taxes       (8,094)       (29,388)       (24,823)       (199,394)         NET LOSS       \$(12,527)       \$(37,425)       \$(65,650)       \$(539,490)         NET LOSS PER SHARE, BASIC       \$(0.07)       \$(0.22)       \$(0.38)       \$(3.29)	Selling, general and administrative	130,532	118,758	394,056	333,635
Total operating expenses347,875339,9811,028,0881,530,571LOSS FROM OPERATIONS(13,727)(60,085)(72,747)(713,808)Equity in the loss of BioMarin/Genzyme LLC(253)(104)(996)(374)Interest income3,9761,63310,0314,561Interest expense(10,884)(9,980)(31,043)(29,767)Other income, net2671,7234,282504LOSS BEFORE INCOME TAXES(20,621)(66,813)(90,473)(738,884)Benefit from income taxes(8,094)(29,388)(24,823)(199,394)NET LOSS\$(12,527)\$(37,425)\$(65,650)\$(539,490)NET LOSS PER SHARE, BASIC\$(0.07)\$(0.22)\$(0.38)\$(3.29)	Intangible asset amortization and contingent consideration	3,760	9,654	26,096	(34,318)
LOSS FROM OPERATIONS(13,727)(60,085)(72,747)(713,808)Equity in the loss of BioMarin/Genzyme LLC(253)(104)(996)(374)Interest income3,9761,63310,0314,561Interest expense(10,884)(9,980)(31,043)(29,767)Other income, net2671,7234,282504LOSS BEFORE INCOME TAXES(20,621)(66,813)(90,473)(738,884)Benefit from income taxes(8,094)(29,388)(24,823)(199,394)NET LOSS\$(12,527)\$(37,425)\$(65,650)\$(539,490)NET LOSS PER SHARE, BASIC\$(0.07)\$(0.22)\$(0.38)\$(3.29)	Impairment of intangible assets		—		599,118
Equity in the loss of BioMarin/Genzyme LLC(253)(104)(996)(374)Interest income3,9761,63310,0314,561Interest expense(10,884)(9,980)(31,043)(29,767)Other income, net2671,7234,282504LOSS BEFORE INCOME TAXES(20,621)(66,813)(90,473)(738,884)Benefit from income taxes(8,094)(29,388)(24,823)(199,394)NET LOSS\$(12,527)\$(37,425)\$(65,650)\$(539,490)NET LOSS PER SHARE, BASIC\$(0.07)\$(0.22)\$(0.38)\$(3.29)	Total operating expenses	347,875	339,981	1,028,088	1,530,571
Interest income3,9761,63310,0314,561Interest expense(10,884)(9,980)(31,043)(29,767)Other income, net2671,7234,282504LOSS BEFORE INCOME TAXES(20,621)(66,813)(90,473)(738,884)Benefit from income taxes(8,094)(29,388)(24,823)(199,394)NET LOSS\$(12,527)\$(37,425)\$(65,650)\$(539,490)NET LOSS PER SHARE, BASIC\$(0.07)\$(0.22)\$(0.38)\$(3.29)	LOSS FROM OPERATIONS	(13,727)	(60,085)	(72,747	) (713,808)
Interest expense(10,884)(9,980)(31,043)(29,767)Other income, net2671,7234,282504LOSS BEFORE INCOME TAXES(20,621)(66,813)(90,473)(738,884)Benefit from income taxes(8,094)(29,388)(24,823)(199,394)NET LOSS\$(12,527)\$(37,425)\$(65,650)\$(539,490)NET LOSS PER SHARE, BASIC\$(0.07)\$(0.22)\$(0.38)\$(3.29)	Equity in the loss of BioMarin/Genzyme LLC	(253)	(104)	(996	) (374 )
Other income, net       267       1,723       4,282       504         LOSS BEFORE INCOME TAXES       (20,621)       (66,813)       (90,473)       (738,884)         Benefit from income taxes       (8,094)       (29,388)       (24,823)       (199,394)         NET LOSS       \$(12,527)       \$(37,425)       \$(65,650)       \$(539,490)         NET LOSS PER SHARE, BASIC       \$(0.07)       \$(0.22)       \$(0.38)       \$(3.29)	Interest income	3,976	1,633	10,031	4,561
LOSS BEFORE INCOME TAXES       (20,621)       (66,813)       (90,473)       (738,884)         Benefit from income taxes       (8,094)       (29,388)       (24,823)       (199,394)         NET LOSS       \$(12,527)       \$(37,425)       \$(65,650)       \$(539,490)         NET LOSS PER SHARE, BASIC       \$(0.07)       \$(0.22)       \$(0.38)       \$(3.29)	Interest expense	(10,884)	(9,980)	(31,043	) (29,767 )
Benefit from income taxes       (8,094 ) (29,388 ) (24,823 ) (199,394 )         NET LOSS       \$(12,527 ) \$(37,425 ) \$(65,650 ) \$(539,490 )         NET LOSS PER SHARE, BASIC       \$(0.07 ) \$(0.22 ) \$(0.38 ) \$(3.29 )	Other income, net	267	1,723	4,282	504
NET LOSS\$(12,527)\$(37,425)\$(65,650)\$(539,490)NET LOSS PER SHARE, BASIC\$(0.07)\$(0.22)\$(0.38)\$(3.29)	LOSS BEFORE INCOME TAXES	(20,621)	(66,813)	(90,473	) (738,884)
NET LOSS PER SHARE, BASIC         \$(0.07)         \$(0.22)         \$(0.38)         \$(3.29)	Benefit from income taxes	(8,094)	(29,388)	(24,823	) (199,394)
	NET LOSS	\$(12,527)	\$(37,425)	\$(65,650	) \$(539,490)
NET LOSS PER SHARE, DILUTED \$(0.07) \$(0.22) \$(0.38) \$(3.30)	NET LOSS PER SHARE, BASIC	\$(0.07)	\$(0.22)	\$(0.38	) \$(3.29 )
	NET LOSS PER SHARE, DILUTED	\$(0.07)	\$(0.22)	\$(0.38	) \$(3.30 )
Weighted average common shares outstanding, basic175,103167,714174,071163,963	Weighted average common shares outstanding, basic	175,103	167,714	174,071	163,963
Weighted average common shares outstanding, diluted 175,103 167,714 174,071 164,216	Weighted average common shares outstanding, diluted	175,103	167,714	174,071	164,216

COMPREHENSIVE LOSS\$(19,303) \$(39,795) \$(99,900) \$(558,365)The accompanying notes are an integral part of these Condensed Consolidated Financial Statements.

## CONDENSED CONSOLIDATED STATEMENT OF STOCKHOLDERS' EQUITY

Nine Months Ended September 30, 2017

(In thousands of U.S. dollars)

(Unaudited)

			Additional	Company Common Stock	Accumulate Other	ed	Total
	Common	stock	Paid-in	Held by	Comprehen Income	siveAccumulated	Stockholders'
	Shares	Amount	Capital	NQDC	(Loss)	Deficit	Equity
Balance at December 31, 2016	172,648	\$ 173	\$4,288,113	\$(14,321)	\$ 12,816	\$(1,520,506)	\$2,766,275
Net loss						(65,650)	(65,650)
Other comprehensive loss	_		_		(34,250	) —	(34,250)
Issuances under equity incentive							
plans, net of tax	1,648	2	7,550				7,552
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		_	_	(152)		_	(152)
Stock-based compensation	_	_	110,606		_	_	110,606
Balance at September 30, 2017	175,495	\$ 176	\$4,435,449	\$(14.473)	\$ (21.434	) \$(1,586,156)	\$2.813.562
The accompanying notes are							, ,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,,

### CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

Nine Months Ended September 30, 2017 and 2016

(In thousands of U.S. dollars)

(Unaudited)

	2017	2016
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	\$(65,650	) \$(539,490)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	59,197	76,805
Non-cash interest expense	23,792	22,276
Accretion of discount on investments	2,162	681
Stock-based compensation	106,678	97,220
(Gain) loss on the sale of equity investments	(3,252	) 2,020
Impairment of intangible assets		599,118
Deferred income taxes	(36,150	) (218,700)
Unrealized foreign exchange (gain) loss	4,348	(10,961)
Non-cash changes in the fair value of contingent acquisition consideration payable	3,382	(56,954)
Other	4,657	1,044
Changes in operating assets and liabilities:		
Accounts receivable, net	(21,598)	) (52,023)
Inventory	(80,885	) (59,802)
Other current assets	(20,787	) (1,556 )
Other assets	(1,030	) (5,002 )
Accounts payable and accrued liabilities	(1,732	) (77,852)
Other long-term liabilities	3,497	(4,451)
Net cash used in operating activities	(23,371	) (227,627)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property, plant and equipment	(159,329)	) (96,806)
Funds held in escrow for the purchase of real property		(8,383)
Maturities and sales of investments	325,678	302,801
Purchase of available-for-sale securities	(609,794)	) (370,393)
Business acquisitions, net of cash acquired		(1,467)
Other	(1,560	) (150 )
Net cash used in investing activities	(445,005)	) (174,398)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from exercises of stock options and the ESPP	46,119	49,498
Taxes paid related to net share settlement of equity awards	(31,863	) (55,241)
Proceeds from public offering of common stock, net		712,938
Proceeds from convertible senior subordinated note offering, net	481,713	_
Payment of contingent acquisition consideration payable	(1,894	) —
Other	(26	) —

Net cash provided by financing activities	494,049	707,195
Effect of exchange rate changes on cash	(2,604	) 5,139
NET DECREASE IN CASH AND CASH EQUIVALENTS	23,069	310,309
Cash and cash equivalents:		
Beginning of period	\$408,330	\$397,040
End of period	\$431,399	\$707,349
SUPPLEMENTAL CASH FLOW DISCLOSURES:		
Cash paid for interest, net of interest capitalized into fixed assets	4,287	4,564
Cash paid for income taxes	21,744	95,163
Stock-based compensation capitalized into inventory	12,077	8,960
Depreciation capitalized into inventory	17,899	13,402
SUPPLEMENTAL CASH FLOW DISCLOSURES FOR NON CASH INVESTING AND		
FINANCING ACTIVITIES:		
Decrease in accounts payable and accrued liabilities related to fixed assets	(25,047	) (13,988
Conversion of convertible debt	22,477	8,924
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 Stat	tements.	

## 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 nine months ended September 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.

## (3) SIGNIFICANT ACCOUNTING POLICIES

There have been no material changes to the Company's significant accounting policies during the nine months ended September 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 nine months ended September 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.

Effective January, 1, 2018, the Company will adopt Accounting Standards Update (ASU) No. 2014-09 (ASU 2014-09), Revenue from Contracts with Customers, as amended (commonly referred to as ASC Topic 606), which 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.

As of September 30, 2017, the Company has not elected to early adopt ASC Topic 606 and plans to adopt the new standard using the modified retrospective 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. As the Company completes its analysis of the accounting for the Company's customer contracts under the new revenue standard, management is assessing 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 ASC Topic 606 will have a material impact on the timing of revenue recognition for the products that are marketed by the Company. Management is still assessing the application of ASC Topic 606 to Aldurazyme revenues earned from Genzyme Corporation (Genzyme).

In January 2016, the Financial Accounting Standards Board (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. Management's assessment indicates that the amendment will not have a significant impact as the Company currently has no significant equity investments, however, the update may have a significant impact in the future. As of September 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 September 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 September 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 may 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.

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 and early adoption is permitted. The Company has

### BIOMARIN PHARMACEUTICAL INC.

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

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

elected to early adopt ASU 2017-09, which did not have a material impact on the Company's Consolidated Financial Statements because the Company's policies had already been in compliance.

In August 2017, the FASB issued ASU No. 2017-12, Derivatives and Hedging (Topic 815): Targeted Improvements to Accounting for Hedging Activities (ASU 2017-12). The amendment changes the recognition and presentation requirements of hedge accounting, including eliminating the requirement to separately measure and report hedge ineffectiveness and presenting all items that affect earnings in the same income statement line as the hedged item. ASU 2017-12 is effective for fiscal years beginning after December 15, 2018, including interim periods within those fiscal years, and early adoption is permitted. Although the Company is currently evaluating the impact that the standard will have on its Consolidated Financial Statements, adoption of the amendment is not expected to have a material impact due to the nature of the Company's hedging activity. As of September 30, 2017, the Company has not elected to early adopt the amendments of ASU 2017-12.

### (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 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 September 30, 2017. See Note 13 to these Condensed Consolidated Financial Statements for additional

### BIOMARIN PHARMACEUTICAL INC.

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

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

discussion regarding fair value measurements of the contingent acquisition consideration payable included on the Company's Condensed Consolidated Balance Sheet.

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

	Three Mor	ths Ended	Nine Mont	hs Ended
	September	30,	September	30,
	2017	2016	2017	2016
Numerator:				
Net loss, basic	\$(12,527)	\$(37,425)	\$(65,650)	\$(539,490)
Less: gain on common stock held by the NQDC	—		_	1,753
Net loss, diluted	\$(12,527)	\$(37,425)	\$(65,650)	\$(541,243)
Denominator:				
Weighted-average common shares outstanding, basic	175,103	167,714	174,071	163,963
Effect of dilutive securities:				
Common shares held by the NQDC		_	_	253
Weighted-average common shares outstanding, diluted	175,103	167,714	174,071	164,216
Net loss per common share, basic	\$(0.07)	\$(0.22)	\$(0.38)	\$(3.29)
Net loss per common share, diluted	\$(0.07)	\$(0.22)	\$(0.38)	\$(3.30)
-				

### 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 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 Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Options to purchase common stock	8,328	9,610	8,328	9,610
Common stock issuable under the 2017 Notes		1,105		1,105
Common stock issuable under the 2018 and 2020 Notes	7,966	7,966	7,966	7,966
Common stock issuable under the 2024 Notes	3,970		3,970	
Unvested restricted stock units	2,923	2,728	2,923	2,728
Common stock potentially issuable for ESPP purchases	365	330	365	330
Common stock held by the NQDC	224	253	224	
Total number of potentially issuable shares	23,776	21,992	23,776	21,739

The potential effect of the capped call transactions with respect to 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) was excluded from the diluted net income/loss per share as the Company's closing stock price on September 30, 2017 and 2016 did not exceed the conversion price of \$94.15 per share for the 2018 Notes and the 2020 Notes. There is no similar capped call transaction associated with the Company's 0.599% senior subordinated convertible notes due in 2024 (the 2024 Notes). See Note 11 to these Condense Consolidated Financial Statements for information on the Company's debt.

#### (7) AVAILABLE-FOR-SALE SECURITIES

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

Amortized	Gross	Gross	Aggregate Fair
Cost			
	Unrealized	Unrealized	Value at

		•	Holding Losses	September 30, 2017
Corporate debt securities	\$762,462	\$ 413	\$ (1,091	) \$ 761,784
Commercial paper	28,039			28,039
U.S. government agency securitie	es 434,533	2	(980	) 433,555
Foreign and other	18,525	123	(22	) 18,626
Total	\$1,243,559	\$ 538	\$ (2,093	) \$ 1,242,004
		Gross	Gross	Aggregate Fair
		Unrealized Holding	l Unrealiz Holding	
	Amortized Cos	Holding		
Certificates of deposit	Amortized Cos \$ 2,800	Holding	Holding	December 31,
Certificates of deposit Corporate debt securities		Holding Gains	Holding Losses	December 31, 2016 \$ 2,800
	\$ 2,800	Holding t Gains \$ —	Holding Losses \$ —	December 31, 2016 \$ 2,800
Corporate debt securities	\$ 2,800 641,670	Holding t Gains \$ —	Holding Losses \$ —	December 31, 2016 \$ 2,800 ) 639,717
Corporate debt securities Commercial paper	\$ 2,800 641,670 16,075	Holding Gains \$ — 329 —	Holding Losses \$ — (2,282 —	December 31, 2016 \$ 2,800 ) 639,717 16,075

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.

### BIOMARIN PHARMACEUTICAL INC.

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

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

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

	September 30,	December 31,
	2017	2016
Maturing in one year or less	\$ 825,700	\$ 395,940
Maturing after one year through five years	416,304	572,711
Total	\$ 1,242,004	\$ 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 September 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.

#### (8) INTANGIBLE ASSETS

Intangible assets consisted of the following:

	September 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
Accumulated amortization	(104,539)	(83,541)
Net carrying value	\$ 530,957	\$ 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 second quarter of 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.

In July 2017, the Company executed a license agreement and a settlement agreement (the Agreements) with Sarepta Therapeutics (Sarepta) that provide Sarepta with global exclusive rights to our 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

### 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 use of EXONDYS 51 and all future exon-skipping products for the treatment of DMD. Pursuant to the Agreements, Sarepta paid the Company a one-time upfront fee of \$35.0 million, which was recognized as license revenue. Under the Agreements, Sarepta may pay certain additional regulatory and commercial milestone fees for exons 51, 45, 53 and possibly on future exon-skipping products to the Company if certain development and sales milestones are achieved. Additionally, Sarepta will pay the Company royalties based on 5% of net sales in the U.S. through the end of 2023 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.

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:

	September 30, 2017	December 31, 2016
Building and improvements	\$ 636,697	\$ 510,805
Manufacturing and laboratory equipment	284,269	242,899
Computer hardware and software	139,241	129,506
Leasehold improvements	43,900	44,184
Furniture and equipment	29,451	27,229
Land improvements	4,881	4,881
Land	62,702	55,412
Construction-in-progress	68,204	126,446
	1,269,345	1,141,362
Accumulated depreciation	(390,721	) (342,594 )
Total property, plant and equipment, net	\$ 878,624	\$ 798,768

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, Ireland.

Depreciation expense for the three and nine months ended September 30, 2017 was \$19.4 million and \$54.8 million, respectively, of which \$6.1 million and \$17.9 million, respectively, was capitalized into inventory. Depreciation expense for the three and nine months ended September 30, 2016 was \$23.2 million and \$56.1 million, respectively, of which \$4.4 million and \$13.4 million, respectively, was capitalized into inventory. Capitalized interest related to the Company's property, plant and equipment purchases for each of the three and nine months ended September 30,

2017 and 2016 was insignificant.

#### (10) SUPPLEMENTAL BALANCE SHEET INFORMATION

Inventory consisted of the following:

	September 30,	December 31,	
	2017	2016	
Raw materials	\$ 48,355	\$ 51,250	
Work-in-process	262,840	167,788	
Finished goods	146,198	136,088	
Total inventory	\$ 457,393	\$ 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 September 30, 2017, the value of the Shanbally qualification campaign was \$25.4 million, which was capitalized into 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.

### BIOMARIN PHARMACEUTICAL INC.

NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS - (Continued)

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

Accounts Payable and Accrued Liabilities consisted of the following:

	September 30,	December 31,
	2017	2016
Accounts payable and accrued operating expenses	\$ 157,024	\$ 191,353
Accrued compensation expense	100,481	109,038
Accrued rebates payable	38,321	34,737
Accrued royalties payable	16,419	15,151
Value added taxes payable	10,577	7,848
Forward foreign currency exchange contracts	14,428	5,201
Deferred Revenue	17,430	985
Other	10,240	6,192
Total accounts payable and accrued liabilities	\$ 364,920	\$ 370,505

### (11) DEBT

#### Convertible Notes

In August 2017, the Company issued \$495.0 million in aggregate principal amount of senior subordinated convertible notes with a maturity date of August 1, 2024. The 2024 Notes were issued to the public at 98% of face value and bear interest at the rate of 0.599% per annum. Interest is payable semi-annually in cash on February 1 and August 1 of each year, beginning February 1, 2018. The 2024 Notes are convertible, at the option of the holder into shares of the Company's common stock. The initial conversion rate for the 2024 Notes is 8.0212 shares per \$1,000 principal amount of the 2024 Notes, which represents a conversion price of approximately \$124.67 per share, subject to adjustment under certain conditions. Following certain corporate transactions, the Company will, in certain circumstances, increase the conversion rate for a holder that elects to convert its 2024 Notes in connection with such corporate transactions by a number of additional shares of the Company's common stock. A holder may convert fewer than all of such holder's 2024 Notes so long as the amount of the 2024 Notes converted is an integral multiple of \$1,000 principal amount. Net proceeds from the offering were \$481.7 million.

The 2024 Notes are senior subordinated, unsecured obligations, and rank (i) subordinated in right of payment to the prior payment in full of any of the Company's existing and future senior debt, (ii) equal in right of payment to any of the Company's existing and future senior subordinated debt, (iii) senior in right of payment to any of the Company's existing and future indebtedness that is expressly subordinated in right of payment to the 2024 Notes, and (iii) effectively subordinated to any of the Company's existing and future secured indebtedness, to the extent of the value of the collateral securing that indebtedness and structurally subordinated to all existing and future indebtedness and other liabilities, including trade payables, and (to the extent the Company is not a holder thereof) preferred equity, if any, of the Company's subsidiaries. Upon the occurrence of a "fundamental change," as defined in the indenture governing the

2024 Notes, the holders may require the Company to repurchase all or a portion of such holder's 2024 Notes for cash at 100% of the principal amount of the 2024 Notes being purchased, plus any accrued and unpaid interest.

In connection with the issuance of the 2024 Notes, the Company recorded a discount on the 2024 Notes of \$9.9 million, which will be accreted and recorded as additional interest expense over the life of the 2024 Notes. During each of the three and nine months ended September 30, 2017, the Company recognized \$0.2 million of debt discount accretion. The Company also incurred \$3.4 million of issuance costs. These costs were deferred and are being amortized over the life of the 2024 Notes and recorded as additional interest expense. During each of the three and nine months ended September 30, 2017, the Company recognized \$0.1 million of amortization of deferred issuance costs related to the 2024 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)

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

	September 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	(16,329	) (27,566 )
Unamortized deferred offering costs	(2,030	) (3,484 )
Convertible Notes due in 2018, net	356,621	343,930
Convertible Notes due in 2020	374,993	374,993
Unamortized discount	(43,593	) (53,239 )
Unamortized deferred offering costs	(3,954	) (4,923 )
Convertible Notes due in 2020, net	327,446	316,831
Convertible Notes due in 2024	495,000	_
Unamortized discount	(9,707	) —
Unamortized deferred offering costs	(3,324	) —
Convertible Notes due in 2024, net	481,969	_
Total convertible debt, net	\$ 1,166,036	\$ 683,239
Fair value of fixed rate convertible debt		
Convertible Notes due in 2017 <sup>(1)</sup>	\$ —	\$ 90,977
Convertible Notes due in 2018 <sup>(1)</sup>	417,956	423,202
Convertible Notes due in 2020 <sup>(1)</sup>	454,334	442,754
Convertible Notes due in 2024 <sup>(1)</sup>	502,960	
Total	\$ 1,375,250	\$ 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 N	Ionths	Nine M	onths	
Ended		Ended		
September 30,		September 30,		
2017	2016	2017	2016	

Coupon interest	\$2,692	\$2,466	\$7,250	\$7,491
Amortization of issuance costs	1,138	826	2,910	2,476
Accretion of debt discount	7,054	6,688	20,883	19,800
Total interest expense on convertible debt	\$10,884	\$9,980	\$31,043	\$29,767

In April 2017, the Company's 1.875% senior subordinated convertible notes due in 2017 (the 2017 Notes) matured, with holders thereof converting \$22.5 million of the 2017 Notes prior to the maturity date into 1,103,704 shares of the Company's common stock. During three and nine months ended September 30, 2016, certain existing holders of the Company's 2017 Notes elected to convert \$2.0 million and \$8.9 million, respectively, in aggregate principal amount of the 2017 Notes into 97,348 and 438,315 shares of the Company's common stock, respectively.

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 (the 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 (the 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 September 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 September 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 September 30, 2017 (notional amounts in millions):

	Number of	Aggregate Notional Amount in Foreign	
Foreign Exchange Contracts	Contracts	Currency	Maturity
Brazilian Reais – Sell	2	33.0	Oct. 2017
Canadian Dollars – Sell	6	6.1	Oct. 2017 - Dec. 2017
Colombian Pesos – Sell	3	15,576.0	Oct. 2017 - Dec. 2017
Euros – Purchase	72	116.7	Oct. 2017 - Sep. 2020
Euros – Sell	304	381.9	Oct. 2017 - Sep. 2020
Total	387		

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 September 2020. Over the next twelve months, the Company expects to reclassify unrealized losses of \$12.9 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 September 30, 2017 (notional amounts in millions):

	Number of	Aggregate Notional Amount in Foreign	
Foreign Exchange Contracts	Contracts	Currency	Maturity
Brazilian Reais – Purchase	1	33.0	Oct. 2017
British Pounds – Sell	1	5.2	Oct. 2017
Euros – Purchase	4	109.8	Oct. 2017
Total	6		

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

	Asset Derivatives September 30, 2017 Balance Sheet Location	Fair Value	Liability Derivatives September 30, 2017 Balance Sheet Location	Fair Value
Derivatives designated as hedging				
instruments:				
Forward foreign currency exchange			Accounts payable and	
contracts	Other current assets	\$ 3,096	accrued liabilities	\$ 14,428
Forward foreign currency exchange				
contracts	Other assets	4,414	Other long- term liabilities	11,380
Total		7,510	-	25,808
Derivatives not designated as				
hedging instruments:				
Forward foreign currency exchange			Accounts payable and	
contracts	Other current assets	1,139	accrued liabilities	
Total		1,139		
Total value of derivative contracts 17		\$ 8,649		\$ 25,808

### 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 Balance Sheet Location	Fair Value	Liability Derivatives December 31, 2016 Balance Sheet Location	Fair Value
Derivatives designated as hedging				
instruments:				
Forward foreign currency exchange			Accounts payable and	
contracts	Other current assets	\$ 13,048	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			Accounts payable and	
contracts	Other current assets	964	accrued liabilities	25
Total		964		25
Total value of derivative contracts		\$ 22,206		\$ 7,543
The effect of the Company's derivat	tive instruments on the C	ondensed Co	onsolidated Financial Statements	for the

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

	Three Mon Ended September 2017		Nine Mor Ended Septembe 2017	
Derivatives Designated as Hedging Instruments:				
Net gain (loss) recognized in accumulated other				
comprehensive loss <sup>(1)</sup> Net gain (loss) reclassified from accumulated	\$(10,569)	\$(1,984)	\$(33,933	) \$(5,857)
other comprehensive income (loss) into earnings (2)	(3,700)	1,486	(489	) 4,616
Net gain recognized in net loss <sup>(3)</sup>	689	9	2,395	5,276
Derivatives Not Designated as Hedging Instruments:				
Net gain (loss) recognized in net loss <sup>(4)</sup>	\$1,655	\$826	\$7,286	\$(2,446)
Net change in the fair value of the effective portion classifie Effective portion classified as Net Product Revenues and SC			r compreh	ensive inco

(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 September 30, 2017 Quoted Price in

#### Active Markets

	For Identical	Significant Other	Significant	
		Observable	Unobservable	
	Assets	Inputo	Inputo	
	(Level	Inputs	Inputs	
	1)	(Level 2)	(Level 3)	Total
Assets:				
Cash and cash equivalents:				
Money market instruments	\$—	\$ 223,845	\$ —	\$223,845
Total cash and cash equivalents		223,845		223,845
Available-for-sale securities:				
Short-term:				
Corporate debt securities		444,966		444,966
Commercial paper	_	28,039		28,039
U.S. government agency securities		334,241		334,241
Foreign and other	_	18,454		18,454
Long-term:				
Corporate debt securities		316,818		316,818
U.S. government agency securities		99,314		99,314
Foreign and other	_	172		172
Total available-for-sale securities		1,242,004		1,242,004
Other current assets:				
NQDC Plan assets		1,226		1,226
Forward foreign currency exchange contract <sup>(1)</sup>	_	4,235		4,235
Restricted investments <sup>(2)</sup>		14,878		14,878
Total other current assets		20,339	—	20,339

Other assets:				
NQDC Plan assets		11,272	—	11,272
Forward foreign currency exchange contract <sup>(1)</sup>		4,414	—	4,414
Total other assets		15,686	—	15,686
Total assets	\$—	\$ 1,501,874	\$ —	\$1,501,874
Liabilities:				
Current liabilities:				
NQDC Plan liability	\$2,635	\$ 1,226	\$ —	\$3,861
Forward foreign currency exchange contract <sup>(1)</sup>		14,428	_	14,428
Contingent acquisition consideration payable			52,609	52,609
Total current liabilities	2,635	15,654	52,609	70,898
Other long-term liabilities:				
NQDC Plan liability	\$18,405	\$ 11,272	—	29,677
Forward foreign currency exchange contract <sup>(1)</sup>		11,380	—	11,380
Contingent acquisition consideration payable			126,790	126,790
Total other long-term liabilities	18,405	22,652	126,790	167,847
Total liabilities	\$21,040	\$ 38,306	\$ 179,399	\$238,745

# 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	Significant Other	Significant	
	Identical	Observable	Unobservable	
	Assets	Inputs	Inputs	
	(Level	Inputs	inputs	
	1)	(Level 2)	(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 <sup>(1)</sup>		14,012		14,012
Restricted investments <sup>(2)</sup>		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 <sup>(1)</sup>		8,194		8,194
Strategic investment <sup>(3)</sup>	4,064			4,064
Total other assets	4,064	17,315		21,379
Total assets	\$4,064	\$ 1,239,466	\$ —	\$1,243,530

Current liabilities:NQDC Plan liability $$2,073$ $$163$ $$ $2,236$ Forward foreign currency exchange contract <sup>(1)</sup> $ 5,201$ $ 5,201$ Contingent acquisition consideration payable $  46,327$ $46,327$ To the excession of the line $2072$ $5264$ $46,327$ $5272$	
Forward foreign currency exchange contract $^{(1)}$ 5,201-5,201Contingent acquisition consideration payable-46,32746,327	
Contingent acquisition consideration payable — 46,327 46,327	
	7
Total current liabilities         2,073         5,364         46,327         53,76	4
Other long-term liabilities:	
NQDC Plan liability 17,303 9,121 — 26,42	4
Forward foreign currency exchange contract <sup><math>(1)</math></sup> — 2,342 — 2,342	
Contingent acquisition consideration payable — — 115,310 115,3	10
Total other long-term liabilities         17,303         11,463         115,310         144,0	76
Total liabilities         \$19,376         \$16,827         \$161,637         \$197,8	40

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

(2) The restricted investments at September 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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## BIOMARIN PHARMACEUTICAL INC.

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

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

(3) The Company 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 nine months ended September 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	
Milestone payments to former		
Huxley Pharmaceuticals, Inc.		
shareholders	(3,500	)
Reversal of contingent		
liability related to revised		
estimate of Firdapse FDA		
acceptance and approval		
milestones	(4,245	)
	7,627	

Changes in the fair value of	
other contingent acquisition	
consideration payable	
Foreign exchange	
remeasurement of Euro	
denominated contingent	
acquisition consideration	
payable	17,880
Contingent acquisition	
consideration payable at	
September 30, 2017	\$ 179,399

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 September 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 on a straight-line basis over a four-year period after the grant date. RSUs granted to directors

## BIOMARIN PHARMACEUTICAL INC.

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

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

generally vest in full one year after the grant date. Shares formerly reserved for future issuance under the 2006 Share Incentive Plan 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. 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		Nine Months Ended		
	September 30,		September 30,		
	2017	2016	2017	2016	
Expected volatility	38 - 40%	38 - 40%	38 - 40%	36 - 44%	
Dividend yield	0.0%	0.0%	0.0%	0.0%	
Expected life				5.0 - 8.1	
	4.9 – 6.6 years	5.0 - 6.7 years	4.9 – 6.6 years	years	
Risk-free interest rate				1.1 –	
	1.8 - 2.1%	1.1 - 1.4%	1.8 - 2.2%	2.1%	

During the nine months ended September 30, 2017, the Company granted options to purchase 785,300 shares of common stock with a weighted-average fair value of \$36.11 per share.

The Company did not issue any new stock purchase rights under the ESPP during the three months ended September 30, 2017.

# 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 closing price of the Company's common stock on that date, ratably over the period during which the vesting restrictions lapse. During the nine months ended September 30, 2017, the Company granted 1,312,350 RSUs with service-based vesting conditions with a weighted-average fair value of \$87.82 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, yest 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 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 closing 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 \$1.2 million and \$2.8 million of compensation expense related to these awards during the three and nine months ended September 30, 2017, respectively.

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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)

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 \$0.9 million and \$3.2 million of compensation expense related to these awards during the three and nine months ended September 30, 2017, respectively. The Company recognized \$1.0 million and \$2.1 million of compensation expense related to these awards during the three and nine months ended September 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 \$0.5 million and \$1.7 million of compensation expense related to these awards during the three and nine months ended September 30, 2017, respectively. The Company recognized \$0.6 million and \$1.8 million of compensation expense related to these awards during the three and nine months ended September 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 Mo	onths	Nine Mon	ths
	Ended		Ended	
	Septembe	er 30,	September	: 30,
	2017	2016	2017	2016
Cost of sales	\$3,007	\$2,092	\$7,803	\$5,943
R&D	13,832	14,165	39,973	42,929
SG&A	19,064	16,645	58,902	48,348
Total stock-based compensation expense	\$35,903	\$32,902	\$106,678	\$97,220

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 not significant.

Stock-based compensation expense of \$4.5 million and \$12.1 million was capitalized into inventory for the three and nine months ended September 30, 2017, respectively, compared to stock-based compensation expense of \$3.5 million and \$9.0 million that was capitalized into inventory for the three and nine months ended September 30, 2016, respectively. Capitalized stock-based compensation is recognized as cost of sales when the related product is sold.

## BIOMARIN PHARMACEUTICAL INC.

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

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

## (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 nine months ended September 30, 2017 and 2016.

	Three Mo Ended Septembe		Nine Months Ended September 30,		Consolidated Statement of
Details about AOCI Components	2017	2016	2017	2016	Comprehensive Loss Classification
Gains (losses) on cash flow hedges:					•
Forward foreign currency exchange					
contracts	\$(4,643)	\$1,436	\$18	\$4,036	Net product revenues
Forward foreign currency exchange					
contracts	943	50	(507)	4,874	SG&A
Total gain (loss) on cash flow hedges	(3,700)	1,486	(489)	8,910	
Gain (loss) on sale of available-for-sale					
		7	2 252	(2,020)	Other
securities	—	7	3,252	,	Other income
Income tax effect of the above	—	(2)	(1,176)	735	Benefit from income taxes
	\$(3,700)	\$1,491	\$1,587	\$7,625	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 nine months ended September 30, 2017 and 2016.

	Unrealized	nths Ended Septemb	er 30, 2017	
	Gains (Losses)	Unrealized Gains		
	on Cash	(Losses) on	Foreign	
	Flow	Available-for-Sale	Currency	
	Hedges	Securities	Items	Total
AOCI balance at June 30, 2017	\$(13,569)	\$ (1,080	)\$(9	) \$(14,658)
Other comprehensive income (loss) before	(10,569)	147	2	(10,420)

reclassifications					
Less: net loss reclassified from AOCI	(3,700)				(3,700)
Tax effect	—	(56	)		(56)
Net current-period other comprehensive income (loss)	(6,869)	91		2	(6,776)
AOCI balance at September 30, 2017	\$(20,438) \$	(989	)\$	(7	) \$(21,434)

		Unrealize Gains (Losses)	onths Ended Septen ed Unrealized Gains (Losses) on		30, 20 oreign	16
		Flow	Available-for-Sale	Cu	ırrenc	У
		Hedges	Securities	Ite	ems	Total
	AOCI balance at June 30, 2016	\$2,305	\$ 2,231	\$	(8	) \$4,528
	Other comprehensive income (loss) before					
	reclassifications	(1,984)	1,738		(1	) (247 )
	Less: gain reclassified from AOCI	1,486	7			1,493
	Tax effect		(630	)		(630)
	Net current-period other comprehensive income (loss)	(3,470)	1,101		(1	) (2,370)
24	AOCI balance at September 30, 2016	\$(1,165)	\$ 3,332	\$	(9	) \$2,158

# BIOMARIN PHARMACEUTICAL INC.

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

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

AOCI balance at December 31, 2016	Nine Mon Unrealized Gains (Losses) on Cash Flow Hedges \$13,006	ths Ended Septemb d Unrealized Gains (Losses) on Available-for-Sale Securities \$ (178	Foreign	Total ) \$12,816
Other comprehensive income (loss) before				
reclassifications	(33,933)	1,981	5	(31,947)
Less: gain (loss) reclassified from AOCI	(489)	3,252		2,763
Tax effect		460		460
Net current-period other comprehensive income (loss)	(33,444)	(811	) 5	(34,250)
AOCI balance at September 30, 2017	\$(20,438)	\$ (989	) \$ (7	) \$(21,434)
	Nine Mon Unrealized Gains (Losses) on Cash Flow Hedges	ths Ended Septemb d Unrealized Gains (Losses) on Available-for-Sale Securities	Foreign	7 Total
AOCI balance at December 31, 2015	\$13,602	\$ 7,441	\$ (10	) \$21,033
Other comprehensive income (loss) before				
reclassifications	(5,857)	(8,477	) 1	(14,333)
Less: gain (loss) reclassified from AOCI	8,910	(2,020	) —	6,890
Tax effect		2,348		2,348
Net current-period other comprehensive income (loss)	(14,767)		) 1	(18,875)
AOCI balance at September 30, 2016	\$(1,165)	\$ 3,332	\$ (9	) \$2,158

#### (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			Nine Ende					
	-	September 30, Septemb					mber 30,		
	2017 2016				2017		2016		
United States	43	%	38	%	39	%	38	%	
Europe	22	%	23	%	21	%	23	%	
Latin America	9	%	14	%	13	%	13	%	
Rest of world	19	%	16	%	20	%	19	%	
Total net product revenue marketed by the Company	93	%	91	%	93	%	93	%	
Aldurazyme net product revenues marketed by Genzyme	7	%	9	%	7	%	7	%	
Total net product revenues	100	%	100	%	100	%	100	%	

## BIOMARIN PHARMACEUTICAL INC.

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

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

The following table illustrates the percentage of the Company's consolidated net product revenues attributed to the Company's largest customers for the three and nine months ended September 30, 2017 and 2016.

	Three Months				Nine Months				
	Ended				Ended				
	September 30,				September 30,				
	2017	'	2016	)	2017	7	2016	)	
Customer A	20	%	19	%	18	%	19	%	
Customer B	16	%	13	%	14	%	13	%	
Customer C	11	%	10	%	10	%	10	%	
Total	47	%	42	%	42	%	42	%	

On a consolidated basis, the Company's two largest customer accounts receivable balances accounted for 20% and 17% of the September 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 September 30, 2017, and December 31, 2016, the accounts receivable balance for Genzyme included \$17.8 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 September 30,		Nine Mont September	30,	
	2017	2016	2017	2016	
Net product revenues by product:					
Aldurazyme	\$22,341	\$23,751	\$61,681	\$58,819	
Brineura	3,107	_	3,361		
Firdapse	5,086	4,981	14,051	13,685	
Kuvan	105,837	90,899	300,127	257,806	
Naglazyme	72,083	77,728	238,392	221,575	
Vimizim	90,298	80,903	299,256	260,310	
Total net product revenues	\$298,752	\$278,262	\$916,868	\$812,195	

## 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 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 Mor	nths Ended	Nine Months Ended		
	September 30,		September	30,	
	2017	2016	2017	2016	
Total revenues by geographic region:					
United States	\$151,010	\$130,356	\$425,107	\$365,674	
Europe	97,825	62,821	228,775	187,328	
Latin America	26,356	38,789	121,824	106,803	
Rest of world	58,957	47,930	179,635	156,958	
Total revenues	\$334,148	\$279,896	\$955,341	\$816,763	

#### (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 December 23, 2016, from Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL), notifying it that DRL had filed an abbreviated new drug application (ANDA) seeking approval of a proposed generic version of Kuvan (sapropterin dihydrochloride) 100 mg oral powder prior to the expiration of the Company's patents listed in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations (the Orange Book). The Company filed a lawsuit alleging patent infringement against DRL. In August 2017, the Company entered into a settlement agreement with DRL (the DRL Powder Settlement Agreement) that resolved the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral powder. Under the terms of the DRL Powder 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 in oral powder form in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances.

The Company also received two separate paragraph IV notice letters, dated January 14, 2016 and January 22, 2015, from Par Pharmaceutical, Inc. (Par), notifying it that Par had filed an ANDA seeking approval of proposed generic versions of Kuvan 100 mg oral powder and Kuvan 100 mg oral tablets, respectively, prior to the expiration of the Company's patents listed in the FDA's Orange Book. The Company filed two lawsuits alleging patent infringement against Par (the lawsuit against Par pertaining to the proposed generic version of Kuvan 100 mg oral tablets was filed together with Merck & Cie), and the two Par cases were consolidated. 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; October 1, 2020 if Par is 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; October 1, 2020 if Par is 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; October 1, 2020 if Par is 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; October 1, 2020 if Par is 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

The Company also received a paragraph IV notice letter, dated October 3, 2014, from DRL notifying it that DRL 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

## BIOMARIN PHARMACEUTICAL INC.

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

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

listed in the FDA's Orange Book. The Company, together with Merck & Cie, filed a lawsuit alleging patent infringement against DRL. 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.

## **Contingent Payments**

As of September 30, 2017, the Company is subject to contingent payments totaling approximately \$600.2 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, \$218.3 million (or €185 million based on the exchange rate of 1.18 USD per Euro in effect on September 30, 2017) relates to the Merck PKU Business acquisition and \$53.3 million relates to programs that are no longer being developed. See Note 13 to these Condensed Consolidated Financial Statements for further information regarding the Company's contingent acquisition consideration payable.

As of September 30, 2017, the Company has recorded a total of \$179.4 million of short-term and long-term contingent acquisition consideration payable on its Condensed Consolidated Balances Sheet, of which \$52.6 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 September 30, 2017, these commitments for the next five years were approximately \$41.5 million. The amounts primarily represent minimum purchase requirements for active pharmaceutical ingredients and post-marketing commitments related to the Company's approved products.

#### (19) 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 nine months ended September 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 nine months ended September 30, 2017 and 2016.

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 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 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 October 2017, we announced that the FDA had completed its review of the Investigational New Drug (IND) application for valoctocogene roxaparvovec (formerly referred to as BMN 270), an investigational gene therapy treatment for severe hemophilia A, and concluded that we could proceed with its clinical development. The IND application included 52-week data at the 6e13 vg/kg dose and the protocol for the Phase 3 study using the 6e13 vg/kg dose. We expect to file for approval of valoctocogene roxaparvovec with 52-week data from the Phase 3 studies. The protocol for the second Phase 3 study using the 4e13 vg/kg dose has also been submitted to the FDA. The FDA granted valoctocogene roxaparvovec Breakthrough Therapy Designation in October 2017. The FDA's Breakthrough Therapy Designation is intended to facilitate and expedite development and review of new drugs to address unmet medical need in the treatment of a serious condition. We also announced that the Phase 3 Clinical Trial Application was approved by the United Kingdom Medicines and Healthcare Products Regulatory Agency (MHRA). In July 2017, we announced that 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. We expect to initiate the global Phase 3 program in the fourth quarter of 2017.

In October 2017, we announced the selection of our next drug development candidate, BMN 290, a selective chromatin modulation therapy intended for treatment of Friedreich's Ataxia (FA), a rare autosomal recessive disorder that results in disabling neurologic and cardiac progressive decline. Currently, there are no approved disease modifying therapies for FA. BMN 290 is a second-generation compound derived from a compound acquired from Repligen Corporation (Repligen) that had human clinical data demonstrating increases in frataxin in FA patients. BMN 290 was selected for its favorable penetration into the central nervous system and cardiac target tissues, and its preservation of the selectivity of the original Repligen compound. We expect to submit the IND application in the second half of 2018.

In October 2017, updated results on the open-label Phase 2 study of vosoritide demonstrated a sustained increase in annualized growth velocity that was accompanied by sustained improvements over time in height compared to age- and gender-matched unaffected children as measure by z-scores. In addition, continued improvement over time in proportionality as measured by a ratio of the upper and lower body measurements, or U/L ratio, was demonstrated. Our on-going Phase 3 trial for vosoritide for the treatment of children with achondroplasia is a randomized, placebo-controlled study of vosoritide in approximately 110 children with achondroplasia ages 5-14 for 52 weeks. This study will be

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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 August 2017, the FDA accepted for Priority Review the Biologics License Application (BLA) 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 on existing management. The FDA is not currently planning to hold an advisory committee meeting to discuss the BLA. The Prescription Drug User Fee Act (PDUFA) action date is February 28, 2018. However, the FDA has requested additional Chemistry, Manufacturing, and Controls (CMC) information, which we expect, when submitted, will be classified as a major amendment and result in a three month extension of the PDUFA date. We also intend to submit a Marketing Authorization Application to the EMA in the first quarter of 2018.

In August 2017, we entered into a settlement agreement with Dr. Reddy's Laboratories, Inc. and Dr. Reddy's Laboratories, Ltd. (collectively, DRL) that resolves the patent litigation with DRL in the U.S. related to Kuvan 100 mg oral powder. Under the terms of the agreement, we granted DRL a non-exclusive license to our patents related to Kuvan to allow DRL to market a generic version of sapropterin dihydrochloride in oral powder form in 100 mg and 500 mg packet formulations in the U.S. for the indications approved for Kuvan beginning on October 1, 2020, or earlier under certain circumstances.

In July 2017, we executed a license agreement and a settlement agreement (the Agreements) with Sarepta Therapeutics (Sarepta) that provide Sarepta with global exclusive rights to our 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. Pursuant to the Agreements, Sarepta paid us a one-time upfront fee of \$35.0 million, which we recognized as license revenue. Under the Agreements, Sarepta may pay certain additional regulatory and commercial milestone fees for exons 51, 45, 53 and possibly on future exon-skipping products to us if certain development and sales milestones are achieved. Additionally, Sarepta will pay us royalties based on 5% of net sales in the U.S. through the end of 2023 and 8% of net sales through September 30, 2024 in the EU and in other countries where certain of our patents exist. We retained the right to convert the license to a co-exclusive right in the event we decide to proceed with an exon-skipping therapy for DMD.

In July 2017, we commissioned our commercial gene therapy manufacturing facility, located in Novato, California, and began Good Manufacturing Practices (GMP) production of valoctocogene roxaparvovec to support clinical development activities and anticipated commercial demand. This facility is capable of supporting the manufacturing of product for approximately 2,000 patients per year, and the production process was developed in accordance with International Conference on Harmonisation guidance for Pharmaceuticals for Human Use facilitating worldwide registration with health authorities.

In June 2017, the European Commission granted marketing authorization for Brineura in the European Union (EU) to treat children with CLN2 disease. The marketing authorization for Brineura includes all 28 countries of the EU, Norway, Iceland and Liechtenstein. Brineura is the first treatment approved in the EU for the treatment of CLN2 disease. We immediately began marketing Brineura in the EU and began shipping the product within the EU in July 2017.

In April 2017, the United States (U.S.) Food and Drug Administration (FDA) approved Brineura to treat children with CLN2 disease. Brineura is the first treatment approved in the U.S. to slow the progression of loss of ambulation in children with CLN2 disease. We immediately began marketing Brineura in the U.S., and began shipping the product within the U.S. in June 2017.

In April 2017, we entered into a settlement agreement with Par Pharmaceutical (Par) that resolves patent litigation in the U.S. with Par 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 granted Par a non-exclusive license to our 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.

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. In September 2017, we announced interim data from the dose escalation portion of a Phase 1/2 trial of BMN 250. We

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continued to observe that BMN 250 reduced heparan sulfate levels to normal range in cerebral spinal fluid of MPS IIIB patients and observed liver size decreases and Development Quotient stabilization or improvement. We reported total revenues of \$334.1 million \$955.3 million for the three and nine months ended September 30, 2017, respectively, compared to \$279.9 million and \$816.8 million for the three and nine months ended September 30, 2016, respectively. Financial Highlights

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

	Three Months Ended September 30,		Nine Months Ended	
			September 30,	
	2017	2016	2017	2016
Total revenues	\$334.1	\$279.9	\$955.3	\$816.8
Cost of sales	59.5	50.7	165.8	145.5
Research and development (R&D) expense	154.1	160.8	442.1	486.7
Selling, general and administrative (SG&A) expense	130.5	118.8	394.1	333.6
Intangible asset amortization and contingent consideration				

expense