BioMarin Announces First Quarter 2017 Financial Results

- First Quarter 2017 Total BioMarin Revenues Increase 28% Year over Year to $304 million

- GAAP Net Loss Reduced to $16 million - Non-GAAP Income of $35 million Reported in the First Quarter 2017

SAN RAFAEL, Calif., May 4, 2017 /PRNewswire/ --

Financial Highlights (in millions of U.S. dollars, except per share data, unaudited)

	Three Months Ended March 31,		
	2017	**2016**	**% Change**
Total BioMarin Revenues	$ 304	$ 237	28 %
Aldurazyme Net Product Revenues	19	16	19 %
Kuvan Net Product Revenues	92	77	19 %
Naglazyme Net Product Revenues	81	65	25 %
Vimizim Net Product Revenues	106	73	45 %
GAAP Net Loss	$ (16)	$ (83)	
GAAP Net Loss per Share - Basic and Diluted	$ (0.09)	$ (0.51)	
non-GAAP Income (Loss) (1)	$ 35	$ (29)	
	March 31, 2017	**December 31, 2016**	
Cash, cash equivalents and investments	$ 1,212	$ 1,362	

(1) Non-GAAP income (loss) is defined by the Company as reported GAAP net income (loss), excluding net interest expense, provision for (benefit from) income taxes, depreciation expense, amortization expense, stock-based compensation expense, contingent consideration expense and certain other specified items as detailed below. Refer to Non-GAAP Information beginning on page 8 of this press release for a complete discussion of the Company's non-GAAP financial information and reconciliations to the comparable GAAP reported information.

BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) today announced financial results for the first quarter ended March 31, 2017. For the quarter ended March 31, 2017, GAAP net loss was $16 million, or $0.09 per basic and diluted share, compared to GAAP net loss of $83 million, or $0.51 per basic and diluted share for the quarter ended March 31, 2016. The change in GAAP net loss year over year was primarily due to higher gross margins from net product sales and lower research and development expenses from the discontinuance of the Kyndrisa and reveglucosidase alfa programs in 2016, partially offset by higher selling, general and administrative expenses.

Non-GAAP income for the first quarter ended March 31, 2017 was $35 million, compared to non-GAAP loss of $29 million for the quarter ended March 31, 2016. Non-GAAP income (loss) is defined by the Company as reported GAAP net income (loss), excluding net interest expense, provision for (benefit from) income taxes, depreciation expense, amortization expense, stock-based compensation expense, contingent consideration expense and certain other specified items as detailed below. Refer to Non-GAAP Information beginning on page 8 of this press release for a complete discussion of the Company's non-GAAP financial information and reconciliations to the comparable GAAP reported information.

Total BioMarin Revenues were $304 million for the first quarter of 2017, an increase of 28% compared to the same period in 2016. Vimizim net product revenues increased to $106 million in the quarter, a 45% year over year increase, primarily driven by 31% growth in new patients on Vimizim therapy year over year. For the first quarter of 2017, Naglazyme net product revenues increased to $81 million, an increase of 25% year over year. The number of patients on Naglazyme therapy continue to show growth with an increase of 9% year over year. For the first quarter of 2017, Kuvan net product revenues increased to $92 million, a 19% year over year increase. Growth was driven by an 8% increase in the number of patients on Kuvan therapy in the U.S. and the completion of the transition of the ex-North American territories acquired in 2016.

As of March 31, 2017, BioMarin had cash, cash equivalents and investments totaling $1.2 billion, as compared to $1.4 billion on December 31, 2016.

Commenting on the quarter, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "We begin 2017 from a position of strength across all facets of the business including regulatory actions, financial results and pipeline development. The recent FDA approval and positive CHMP opinion of Brineura marks a significant regulatory accomplishment for the Company and more importantly, a turning point for families living with CLN2 disease who now have an approved treatment option. The speed with which global health authorities reviewed our applications both in the U.S. and Europe speaks to the value our innovative medicines provide patients. Following the recent CHMP positive opinion, we look forward to a potential approval in Europe later in the second quarter. With the recent FDA approval, we are now executing on our commercial launch of Brineura in the U.S." Mr. Bienaimé continued, "BioMarin's robust commercial business continues to support our investment in research and development programs and the next generation of potential products. All of the commercial products we market delivered double-digit growth year over year, and while we expect to incur a GAAP net loss for 2017, we are on a path toward achieving non-GAAP income for the full-year 2017, as planned."

Revenues (in millions of U.S. dollars, unaudited)

Total BioMarin Revenues

	Three Months Ended March 31,			
	2017	**2016**	**$ Change**	**% Change**
Aldurazyme	$ 19	$ 16	$ 3	19 %
Firdapse	4	4	-	0 %
Kuvan (1)	92	77	15	19 %
Naglazyme (2)	81	65	16	25 %
Vimizim (2)	106	73	33	45 %
Net product revenues	302	235	67	29 %
Royalty and other revenues	2	2	-	
Total BioMarin Revenues	$ 304	$ 237	$ 67	28 %

(1) Kuvan revenue growth was driven by an 11% increase in the number of patients on Kuvan therapy in the U.S. and the completion of the transition of the ex-North American territories acquired in 2016.

(2) Naglazyme and Vimizim net product revenues experience quarterly fluctuations primarily due to the timing of government ordering patterns in certain countries.

Details of Net Product Revenues Attributable to Aldurazyme

	Three Months Ended March 31,			
	2017	2016	$ Change	% Change
Aldurazyme revenue reported by Genzyme	$ 55	$ 53	$ 2	4 %

	Three Months Ended March 31,		
	2017	2016	$ Change
Royalties earned from Genzyme	$ 25	$ 22	$ 3
Net product transfer revenues [3]	(6)	(6)	-
Total Aldurazyme net product revenues	$ 19	$ 16	$ 3

(3) To the extent units shipped to third party customers by Genzyme exceed BioMarin inventory transfers to Genzyme, BioMarin will record a decrease in net product revenues from the amounts payable to BioMarin for the amount of previously recognized product transfer revenue. If BioMarin inventory transfers exceed units shipped to third party customers by Genzyme, BioMarin will record incremental net product transfer revenues for the period. Positive net product transfer revenues result in the period if BioMarin transferred more units to Genzyme than Genzyme sold to third-party customers.

2017 Financial Guidance (unchanged)

Revenue Guidance ($ in millions)

Item	2017 Guidance
Total BioMarin Revenues	$1,250 to $1,300
Kuvan Net Product Revenues	$380 to $410
Naglazyme Net Product Revenues	$300 to $330
Vimizim Net Product Revenues	$400 to $430

Select Income Statement Guidance ($ in millions, except percentages)

Item	2017 Guidance
Cost of Sales (% of Total BioMarin Revenues)	17.5% to 18.5%
Research and Development Expense	$620 to $650
Selling, General and Admin. Expense	$520 to $550
GAAP Net Loss	$(140) to $(180)
non-GAAP Income	$30 to $70

Key Program Updates

- **Brineura for CLN2, late-infantile form of Batten disease:** On April 27, 2017, the Company announced that the U.S.

Food and Drug Administration (FDA) had approved Brineura, the first approved treatment for any form of Batten Disease. The Company has begun its commercial launch of Brineura in the U.S.

On April 21, 2017, the Company received a positive opinion for approval of Brineura from the Committee for Medicinal Products for Human Use (CHMP) under revised guidelines for Accelerated Assessment. The CHMP's recommendation is now referred to the European Commission (EC), which is expected to render its final decision by the second quarter of 2017. The EC typically adheres to the recommendation of the CHMP, but is not obligated to do so. If approved by the EC, BioMarin will receive marketing authorization for Brineura in all 28 countries of the European Union, Norway, Iceland and Liechtenstein.

- **BMN 270 gene therapy product for hemophilia A:** During the first quarter, the Company dosed three additional subjects with the 4×10^{13} vg/kg dose of BMN 270 in the ongoing Phase 1/2 study. Currently, six subjects have received the 4×10^{13} vg/kg dose and seven have received the 6×10^{13} vg/kg dose.

  The Company intends to provide the next program update at the International Society of Thrombosis and Haemostasis (ISTH) 2017 Congress in Berlin in July and start the potentially registration enabling Phase 2b study in 3Q 2017. In addition, the company is expected to commission its commercial gene therapy manufacturing facility by mid-2017.

- **Kuvan® for phenylketonuria:** On April 13, 2017, the Company announced that it had entered into a settlement agreement with Par Pharmaceutical that resolves patent litigation in the United States (U.S.) related to BioMarin's Kuvan (sapropterin dihydrochloride) 100mg oral tablets and powder for oral solution in 100mg packets.

  Under the terms of the settlement, BioMarin will grant Par a non-exclusive license to its patents related to Kuvan to allow Par to market a generic version of sapropterin dihydrochloride 100mg tablets and powder for oral solution in 100mg and 500 mg sachets in the U.S. for the indications approved for Kuvan beginning October 1, 2020 if Par is entitled to the statutory 180-day first filer exclusivity period; April 1, 2021 if Par is not entitled to the statutory 180-day first filer exclusivity period; or earlier under certain circumstances. Additional details of the agreement remain confidential. BioMarin continues to vigorously enforce its intellectual property related to Kuvan. BioMarin holds patents in Europe related to Kuvan that are valid until at least 2024.

- **Vosoritide for achondroplasia:** In December 2016, the Company announced that it had initiated a global Phase 3 study for vosoritide, an analog of C-type Natriuretic Peptide (CNP), in children with achondroplasia, the most common form of dwarfism. The first child enrolled in the study was at a site in Australia. The primary endpoint of the study is the change in growth velocity from baseline over one year in children treated compared to placebo. Following discussions with global health authorities, the company also 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.

  The Phase 3 study is a randomized, placebo-controlled study of vosoritide in approximately 110 children with achondroplasia ages 5-14 for 52 weeks. The study will be followed by a subsequent open-label extension. Children in this study will have completed a minimum six-month baseline study to determine their respective baseline growth velocity prior to entering the Phase 3 study. Vosoritide is being tested in children in the age range where their growth plates are still open. This is approximately 25 percent of people with achondroplasia.

- **Pegvaliase for phenylketonuria (PKU):** Based on the supportive data results reported in 2016, the Company plans to submit a Biologics License Application to the FDA in the second quarter of 2017. Last year, the Company announced pivotal results for the Phase 3 PRISM-2 study (formerly referred to as 165-302) with pegvaliase demonstrating that the primary endpoint of change in blood phe compared with placebo ($p<0.0001$) had been met. The pegvaliase treated group maintained mean blood phe levels at 527.2 umol/L compared to their Randomized Discontinuation Trial (RDT)

baseline of 503.9 umol/L, whereas the placebo treated group mean blood phe levels increased to 1385.7 umol/L compared to their RDT baseline of 536.0 umol/L. The treatment effect demonstrated in this study represents an approximately 62% improvement in blood phe compared to placebo.

- **BMN 250 for MPS IIIB (Sanfilippo Syndrome, Type B):** In the first quarter, the Company shared preliminary data from the Phase 1/2 program demonstrating that BMN 250 reduced heparan sulfate, a biomarker in the cerebrospinal fluid (CSF), in the brains of affected children. Since sharing these results with 30mg/weekly dose, patients have been safely escalated to the highest dose of 300mg weekly. The study will now move to the expansion phase with the highest dose and measuring changes in neurocognitive function in children with this rapid and progressive neurodegenerative disease.

Conference Call Details

BioMarin will host a conference call and webcast to discuss first quarter 2017 financial results today, Thursday, May 4, 2017 at 4:30 p.m. ET. This event can be accessed on the investor section of the BioMarin website at www.biomarin.com.

U.S. / Canada Dial-in Number: 866.502.9859
International Dial-in Number: 547.990.1362
Conference ID: 2424152

Replay Dial-in Number: 855.859.2056
Replay International Dial-in Number: 404.537.3406
Conference ID: 2424152

About BioMarin
BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare and ultra-rare genetic diseases. The Company's portfolio consists of six approved products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.biomarin.com.

Forward-Looking Statement
This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about: the expectations of total BioMarin revenues, net product revenues and expenses for BioMarin's commercial products, GAAP net loss, non-GAAP income (loss) and other specified income statement guidance; the potential revenues and expenses related to BioMarin's product candidates, including BMN 270, pegvaliase, vosoritide and BMN 250; the financial performance of BioMarin as a whole; the timing of BioMarin's clinical studies and trials and announcements of data from those studies and trials; the continued clinical development and commercialization of BioMarin's commercial products and product candidates; the possible approval and commercialization of BioMarin's product candidates, including the launch of Brineura in the U.S. and potential approval of Brineura in Europe; the timing of the commissioning of the Company's commercial gene therapy manufacturing facility; and actions by regulatory authorities. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: BioMarin's success in the commercialization of its commercial products; Genzyme Corporation's success in continuing the commercialization of Aldurazyme; results and timing of current and planned preclinical studies and clinical trials, BioMarin's ability to successfully manufacture its commercial products and product candidates; the content and timing of decisions by the FDA, the European Commission and other regulatory authorities concerning each of the described products and product candidates; the market for each of these products; actual sales of BioMarin's commercial products; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission (SEC), including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's Annual Report on Form 10-K for the year ended December 31, 2016 as such factors may be updated by any subsequent reports. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement,

whether as a result of new information, future events or otherwise.

BioMarin®, Vimizim®, Naglazyme®, Kuvan® and Firdapse® are registered trademarks of BioMarin Pharmaceutical Inc., or its affiliates. Brineura™ and Kyndrisa™ are trademarks of BioMarin Pharmaceutical Inc. Aldurazyme ® is a registered trademark of BioMarin/Genzyme LLC.

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

March 31, 2017 and December 31, 2016

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

	March 31, 2017	December 31, 2016(1)
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 348,234	$ 408,330
Short-term investments	329,821	381,347
Accounts receivable, net (allowance for doubtful accounts: $61 and $73, at March 31, 2017 and December 31, 2016, respectively)	230,250	215,280
Inventory	384,143	355,126
Other current assets	60,340	61,708
Total current assets	1,352,788	1,421,791
Noncurrent assets:		
Long-term investments	533,628	572,711
Property, plant and equipment, net	824,013	798,768
Intangible assets, net	546,172	553,780
Goodwill	197,039	197,039
Deferred tax assets	458,387	446,786
Other assets	26,580	32,815
Total assets	$ 3,938,607	$ 4,023,690
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 272,447	$ 370,505
Short-term convertible debt, net	22,495	22,478
Short-term contingent acquisition consideration payable	45,264	46,327
Total current liabilities	340,206	439,310
Noncurrent liabilities:		
Long-term convertible debt, net	668,437	660,761
Long-term contingent acquisition consideration payable	116,393	115,310

Other long-term liabilities	44,484	42,034
Total liabilities	1,169,520	1,257,415
Stockholders' equity:		
Common stock, $0.001 par value: 250,000,000 shares authorized; 173,304,193 and 172,647,588 shares issued and outstanding as of March 31, 2017 and December 31, 2016, respectively.	173	173
Additional paid-in capital	4,314,793	4,288,113
Company common stock held by Nonqualified Deferred Compensation Plan	(14,103)	(14,321)
Accumulated other comprehensive income	5,020	12,816
Accumulated deficit	(1,536,796)	(1,520,506)
Total stockholders' equity	2,769,087	2,766,275
Total liabilities and stockholders' equity	$ 3,938,607	$ 4,023,690

(1) December 31, 2016 balances were derived from the audited Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2016, filed with the U.S. Securities and Exchange Commission on February 27, 2017.

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

Three Months Ended March 31, 2017 and 2016

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

(Unaudited)

	Three Months Ended March 31,	
	2017	**2016**
REVENUES:		
Net product revenues	$ 302,190	$ 235,357
Royalty and other revenues	1,555	1,379
Total revenues	303,745	236,736
OPERATING EXPENSES:		
Cost of sales	50,006	43,118
Research and development	145,003	158,793
Selling, general and administrative	120,019	105,300
Intangible asset amortization and contingent consideration	8,925	10,442
Total operating expenses	323,953	317,653
LOSS FROM OPERATIONS	(20,208)	(80,917)
Equity in the loss of BioMarin/Genzyme LLC	(523)	(135)
Interest income	3,072	1,571

Interest expense	(10,119)	(9,843)
Other income	3,472	198
LOSS BEFORE INCOME TAXES	(24,306)	(89,126)
Benefit from income taxes	(8,016)	(6,075)
NET LOSS	$ (16,290)	$ (83,051)
NET LOSS PER SHARE, BASIC AND DILUTED	$ (0.09)	$ (0.51)
Weighted average common shares outstanding, basic and diluted	172,710	161,548

Non-GAAP Information

The results presented in this press release for the three months ended March 31, 2017 and 2016 include both GAAP information and non-GAAP information. As used in this release, non-GAAP income (loss) is defined by the Company as GAAP net loss excluding net interest expense, provision for (benefit from) income taxes, depreciation expense, amortization expense, stock-based compensation expense, contingent consideration expense and certain other specified items, as detailed below. In addition, BioMarin includes in this press release the effects of these adjustments on certain components of GAAP net loss for each of the periods presented. In this regard, non-GAAP income (loss) and its components, including non-GAAP Cost of sales, non-GAAP Research and development expenses, non-GAAP Selling, general and administrative expense, non-GAAP Intangible asset amortization and contingent consideration, non-GAAP Other income (expense) and non-GAAP Provision for (benefit from) income taxes are statement of operations line items prepared on the same basis as, and therefore components of, the overall non-GAAP measures.

BioMarin regularly uses both GAAP and non-GAAP results and expectations internally to assess its financial operating performance and evaluate key business decisions related to its principal business activities – the discovery, development, manufacture, marketing and sale of innovative biologic therapies. Because non-GAAP income (loss) and its components are important internal measurements for BioMarin, the Company believes that providing this information in conjunction with BioMarin's GAAP information enhances investors' and analysts' ability to meaningfully compare the Company's results from period to period and to its forward looking guidance, and to identify operating trends in the Company's principal business.

Non-GAAP income (loss) and its components are not meant to be considered in isolation, as a substitute for, or superior to comparable GAAP measures and should be read in conjunction with the consolidated financial information prepared in accordance with GAAP. Investors should note that the non-GAAP information is not prepared under any comprehensive set of accounting rules or principles and does not reflect all of the amounts associated with the Company's results of operations as determined in accordance with GAAP. Investors should also note that these non-GAAP measures have no standardized meaning prescribed by GAAP and, therefore, have limits in their usefulness to investors. In addition, from time to time in the future there may be other items that the Company may exclude for purposes of its non-GAAP measures; likewise, the Company may in the future cease to exclude items that it has historically excluded for purposes of its non-GAAP measures. Because of the non-standardized definitions, the non-GAAP measure as used by BioMarin in this press release and the accompanying tables may be calculated differently from, and therefore may not be directly comparable to, similarly titled measures used by other companies.

The following table presents the reconciliation of GAAP Net Loss to non-GAAP Income (Loss):

Reconciliation of GAAP Net Income (Loss) to non-GAAP Income (Loss)

(In millions of U.S. dollars)

(unaudited)

	Three Months Ended March 31,		Year Ending December 31, 2017
	2017	2016	Guidance
GAAP Net Loss	**$ (16)**	**$ (83)**	**$(180) - $(140)**
Interest expense, net	7	8	35
Benefit from income taxes	(8)	(6)	(60) - (100)
Depreciation expense	12	12	45 - 55
Amortization expense	8	8	30
Stock-based compensation expense	31	30	135 - 155
Contingent consideration expense	1	2	25 - 35
non-GAAP Income (Loss)	$ 35	$ (29)	$30 - $70

The following reconciliation of the GAAP reported to non-GAAP information provides the details of the effects of the non-GAAP adjustments on certain components of the Company's operating results for each of the periods presented.

Reconciliation Of Certain GAAP Reported Information To non-GAAP Information

Three Months Ended March 31, 2017 and 2016

(In millions of U.S. dollars)

(Unaudited)

	Three Months Ended March 31,						
	2017				2016		
		Adjustments				Adjustments	
	GAAP Reported	Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	non-GAAP	**GAAP Reported**	Interest, Taxes, Depreciation and Amortization	Stock-E Compen Contir Conside and C Adjust
Cost of sales	**$ 50**	$ —	$ (2)	$ 48	**$ 43**	$ -	$
Research and development	**145**	(7)	(11)	127	**159**	(6)	
Selling, general and administrative	**120**	(5)	(18)	97	**105**	(6)	
Intangible asset amortization							

and contingent consideration	**9**	(8)	(1)	—	**10**	(8)
Interest expense, net	**(7)**	7	—	—	**(8)**	8
Benefit from income taxes	**(8)**	8	—	—	**(6)**	6
GAAP Net Loss/non-GAAP Income (Loss)	**(16)**	19	32	35	**(83)**	22

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