

BioMarin Announces First Quarter 2016 Financial Results

First Quarter 2016 Total BioMarin Revenue Increases 16.7% Y/Y to \$236.7 million

Vimizim Net Product Revenue Increases 43.5% Y/Y and Contributes \$72.6 million in the First Quarter 2016; Vimizim Full-year Revenue Guidance Increased to \$315 to \$340 million

Kuvan Net Product Revenue Contributes \$76.9 million in the First Quarter 2016; \$60.0 million from North America and \$16.9 million from Newly Acquired ex-North American Territories

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

	Three Months Ended March 31,			
	2016	2015	% Change	
Total BioMarin Revenue	\$ 236.7	\$ 202.9	16.7	%
Vimizim Net Product Revenue	72.6	50.6	43.5	%
Naglazyme Net Product Revenue	65.4	78.2	(16.4))%
Kuvan Net Product Revenue	76.9	50.2	53.2	%
Aldurazyme Net Product Revenue	16.4	18.2	(9.9))%
Non-GAAP Net Loss	\$ (27.2)	\$ (25.4)		
GAAP Net Loss	\$ (85.1)	\$ (67.5)		
GAAP Net Loss per Share - Basic and Diluted	\$ (0.53)	\$ (0.43)		
Cash, cash equivalents and investments	\$ 771.3	\$ 1,018.3		

SAN RAFAEL, Calif., April 28, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) today announced financial results for the first quarter ended March 31, 2016. Non-GAAP net loss was \$27.2 million for the quarter ended March 31, 2016, compared to non-GAAP net loss of \$25.4 million for the first quarter of 2015. GAAP net loss was \$85.1 million, or \$0.53 per basic and diluted share for the first quarter of 2016, compared to GAAP net loss of \$67.5 million, or \$0.43 per basic and diluted share, for the first quarter of 2015.

Total BioMarin Revenue was \$236.7 million for the first quarter of 2016, an increase of 16.7% compared to the same period in 2015. This strong result was driven by year over year growth of 43.5% and 53.2% of Vimizim and Kuvan, respectively. Kuvan revenue from ex-North America territories since BioMarin acquired worldwide rights in January 2016 contributed \$16.9 million and

revenues in North America contributed \$60.0 million in the quarter. Naglazyme patient growth was 8.5% compared to a year ago, the 40th straight quarter since the product was launched in 2005. Naglazyme revenue in the first quarter 2016 was lower than revenue in the first quarter 2015 primarily due to the timing of central government orders from Latin America.

As of March 31, 2016, BioMarin had cash, cash equivalents and investments totaling \$771.3 million, as compared to \$1,018.3 million on December 31, 2015.

Commenting on the quarter, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin said, "Our commercial base business is robust and is expected to generate over one billion dollars in revenues this year. Prospects for new product launches in 2017 increased during the quarter due to positive data readouts for cerliponase alfa and pegvaliase that we expect will lead to two new product filings later this year. In addition, at our recent Research and Development Day for analysts and investors, we highlighted very encouraging preliminary data from our gene therapy product BMN 270 for hemophilia A and robust 12 month data with vosoritide for achondroplasia. If the data from these programs continue to mature as we hope, we believe that these products could each ultimately drive a billion dollars in revenue when commercialized. Finally, we continue to expect to manage this growing business with the goal of achieving non-GAAP break-even or better in 2017 regardless of the regulatory outcome of Kyndrisa in Europe."

Net Product Revenue (in millions of U.S. dollars, unaudited)

Total Revenue

	Three Months Ended March 31,				
	2016	2015	\$ Change	% Change	
Vimizim ⁽¹⁾	\$ 72.6	\$ 50.6	\$ 22.0	43.5	%
Naglazyme ⁽¹⁾	65.4	78.2	(12.8)	(16.4)	%
Kuvan ⁽²⁾	76.9	50.2	26.7	53.2	%
Aldurazyme	16.4	18.2	(1.8)	(9.9)	%
Firdapse	4.1	4.1	-	0.0	%
Net product revenues	235.4	201.3	34.1	16.9	%
Collaborative agreement revenues	0.2	0.4	(0.2)		
Royalty, license and other revenues	1.1	1.2	(0.1)		
Total BioMarin revenues	\$ 236.7	\$ 202.9	\$ 33.8	16.7	%

(1) Vimizim and Naglazyme revenues experience quarterly fluctuations primarily due to the timing

of government ordering patterns in certain countries. The Company does not believe these fluctuations reflect a change in underlying demand.

(2) Growth in North America contributed \$60.0 million in the first quarter with an additional \$16.9 million coming from newly acquired ex-North American territories.

Reconciliation of Aldurazyme Revenues

	Three Months Ended March 31,			
	2016	2015	\$ Change	% Change
Aldurazyme revenue reported by Genzyme	\$ 52.8	\$ 53.4	\$ (0.6)	(1.1)%

	Three Months Ended March 31,		
	2016	2015	\$ Change
Royalties earned from Genzyme	\$ 21.5	\$ 22.3	\$ (0.8)
Net product transfer revenues ⁽³⁾	(5.1)	(4.1)	(1.0)
Total Aldurazyme net product revenues	\$ 16.4	\$ 18.2	\$ (1.8)

(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 revenue from the royalty 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 revenue 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.

2016 Financial Guidance

Revenue Guidance (\$ in millions)

Item	Provided February 25, 2016	Updated April 28, 2016
Total BioMarin Revenues	\$1,050 to \$1,100	Unchanged
Vimizim Net Product Revenue	\$300 to \$330	\$315 to \$340
Naglazyme Net Product Revenue	\$290 to \$320	Unchanged
Kuvan Net Product Revenue	\$320 to \$350	Unchanged

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

Item	Provided February 25, 2016	Updated April 28, 2016
Cost of Sales (% of Total Revenue)	18.0% to 19.0%	Unchanged
Selling, General and Admin. Expense	\$470 to \$490	Unchanged
Research and Development Expense	\$680 to \$720	Unchanged
Non - GAAP Net Loss	\$(75) to \$(100)	Unchanged
GAAP Net Loss	\$(400) to \$(430)	\$(355) to \$(385)*

*GAAP Net Loss guidance updated April 28, 2016 based on impact of final purchase accounting treatment for the PKU franchise acquisition from Merck that closed in the first quarter 2016.

Key Program Updates at R&D Day April 20, 2016

- **BMN 270 gene therapy product for hemophilia A:** The Company provided encouraging preliminary data from an ongoing Phase 1/2 clinical trial with BMN 270, an investigational gene therapy treatment for hemophilia A. A total of eight patients with severe hemophilia A received a single dose of BMN 270, six of whom have been treated at the highest dose of 6×10^{13} vector genomes (VG)/kilogram (kg), and to date, post-treatment follow-up ranges from five to 16 weeks. As stated at R&D Day, patients at the highest dose experienced increasing Factor VIII activity levels ranging between 4% and 60% (as a percentage of normal calculated based on the numbers of International Units (IU) per milliliter of whole blood), with five of six patients treated at the high dose now over 5% and two of six at over 50%. All high dose patients improved from severe to either moderate, mild or normal range in terms of factor levels based on World Federation of Hemophilia criteria. (See BioMarin press release from April 20, 2016 for further details.)
- **Vosoritide for achondroplasia:** The Company provided an update on its Phase 2 study of vosoritide, an analog of C-type Natriuretic Peptide (CNP), in children with achondroplasia, the most common form of dwarfism. After 12 months of daily dosing at 15 $\mu\text{g}/\text{kg}/\text{day}$, the cohort 3 patients (n=10) experienced a 46% or 1.9 cm/year increase in mean annualized growth velocity from baseline (p-value = 0.02). These findings provide evidence of durability of effect consistent with previously presented 6-month data for these patients, which demonstrated an annualized increase of 50% or 2.0 cm/year in mean annualized growth velocity. In addition, 6-month data for 12 patients who were initiated on

a lower dose and switched to 15 µg/kg/day showed an increase of 65% or 2.3 cm/year in mean annualized growth velocity from baseline (p-value = 0.002). (See BioMarin press release from April 20, 2016 for further details.)

- **Cerliponase alfa for CLN2, late-infantile form of Batten disease:** Complete results from the Phase 1/2 study of cerliponase alfa, a recombinant human tripeptidyl peptidase 1 (rhTPP1), for the treatment of patients with late-infantile neuronal ceroid lipofuscinosis type 2 (NCL-2), a form of Batten disease were announced at the WORLD LSD Symposium on March 2, 2016. Based on the robust data results announced at that meeting, the Company shared plans to submit in the U.S. and E.U. for regulatory approval mid-year 2016. (See BioMarin press release from March 2, 2016 for further details.)
- **Pegvaliase for phenylketonuria (PKU):** Pivotal results for the Phase 3 PRISM-2 study (formerly referred to as 165-302) that pegvaliase met the primary endpoint of change in blood Phe compared with placebo (p<0.0001) were announced March 21, 2016. The pegvaliase treated group maintained mean blood Phe levels at 527.2 umol/L compared to their 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. Based on the supportive data results, the Company plans to submit a Biologics License Application (BLA) to U.S. FDA in the second half of 2016. (See BioMarin press release from March 21, 2016 for further details.)

Conference Call Details

BioMarin will host a conference call and webcast to discuss first quarter 2016 financial results today, Thursday, April 28, at 4:30 p.m. ET. This event can be accessed on the investor section of the BioMarin website at www.BMRN.com.

U.S. / Canada Dial-in Number: 877.303.6313

International Dial-in Number: 631.813.4734

Conference ID: 86095021

Replay Dial-in Number: 855.859.2056

Replay International Dial-in Number: 404.537.3406

Conference ID: 86095021

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 five commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.BMRN.com.

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the expectations of revenue and sales related to Vimizim, Naglazyme, Kuvan, Firdapse, and Aldurazyme; the financial performance of the BioMarin as a whole; the timing of BioMarin's clinical trials; the continued clinical development and commercialization of Vimizim, Naglazyme, Kuvan, Firdapse, Aldurazyme and its product candidates; 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: our success in the commercialization of Vimizim, Naglazyme, Kuvan, and Firdapse; Genzyme Corporation's success in continuing the commercialization of Aldurazyme; results and timing of current and planned preclinical studies and clinical trials, our ability to successfully manufacture our products and product candidates; the content and timing of decisions by the U.S. Food and Drug Administration, 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 Vimizim, Naglazyme, Kuvan, Firdapse and Aldurazyme; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's 2015 Annual Report on Form 10-K, and the factors contained in BioMarin's reports on Form 10-Q. 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[®], Naglazyme[®], Kuvan[®], Firdapse[®] and Vimizim[®] are registered trademarks of BioMarin Pharmaceutical Inc., or its affiliates. Kyndrisa[™] is a trademark of BioMarin Pharmaceutical Inc. Aldurazyme[®] is a registered trademark of BioMarin/Genzyme LLC.

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

March 31, 2016 and December 31, 2015

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

	March 31, 2016	December 31, 2015(1)
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	\$ 270,453	\$ 397,040
Short-term investments	186,400	195,579
Accounts receivable, net (allowance for doubtful accounts: \$167 and \$93, at March 31, 2016 and December 31, 2015, respectively)	180,751	164,959
Inventory	296,979	271,683
Other current assets	58,207	60,378
Total current assets	992,790	1,089,639
Noncurrent assets:		
Long-term investments	314,404	425,652
Property, plant and equipment, net	716,916	704,207
Intangible assets, net	1,177,232	683,996
Goodwill	197,039	197,039
Long-term deferred tax assets	243,212	220,191
Other assets	25,400	408,644
Total assets	\$ 3,666,993	\$ 3,729,368
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	290,562	392,511
Short-term contingent acquisition consideration payable	97,449	52,946
Total current liabilities	388,011	445,457
Noncurrent liabilities:		
Long-term convertible debt	668,009	662,286
Long-term contingent acquisition consideration payable	135,275	32,663
Long-term deferred tax liabilities	143,527	143,527
Other long-term liabilities	41,935	44,588
Total liabilities	1,376,757	1,328,521
Stockholders' equity:		
Common stock, \$0.001 par value: 250,000,000 shares authorized at March 31, 2016 and December 31, 2015: 162,243,016 and 161,526,044 shares issued and outstanding at March 31, 2016 and December 31, 2015, respectively	163	162
Additional paid-in capital	3,410,297	3,414,837
Company common stock held by Nonqualified Deferred Compensation Plan	(13,560)	(13,616)
Accumulated other comprehensive income	47	21,033
Accumulated deficit	(1,106,711)	(1,021,569)
Total stockholders' equity	2,290,236	2,400,847
Total liabilities and stockholders' equity	\$ 3,666,993	\$ 3,729,368

(1) December 31, 2015 balances were derived from the audited Consolidated Financial Statements included in the Company's Annual Report on Form 10-K for the year ended December 31, 2015, filed with the U.S. Securities and Exchange Commission on February 29, 2016.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
Three Months Ended March 31, 2016 and 2015

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

(Unaudited)

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

Non-GAAP Loss

The results for the three months ended March 31, 2016 and 2015 include both GAAP net loss and non-GAAP loss. As used in this release, non-GAAP loss is based on GAAP net loss and the guidance for full-year net loss before interest, taxes, depreciation and amortization and further adjusted to exclude certain non-cash stock-based compensation expense, non-cash contingent consideration expense and certain other specified items, as detailed below (non-GAAP loss).

BioMarin believes that the non-GAAP information in this press release is useful to investors, taken in conjunction with BioMarin's GAAP information because it provides additional information regarding the performance of BioMarin's core ongoing business, Naglazyme, Vimizim, Kuvan, Aldurazyme and Firdapse and development of its pipeline. By providing information about both the overall GAAP financial performance and the non-GAAP measures that focus on continuing operations, the Company believes that the additional information enhances investors' overall understanding of the Company's business and prospects for the future. Further, the Company

uses both the GAAP and the non-GAAP results and expectations internally for its operating, budgeting and financial planning purposes.

The following table presents the reconciliation of GAAP to non-GAAP financial metrics:

Reconciliation of GAAP Net Loss to Non-GAAP Loss

(In millions of U.S. dollars)

(Unaudited)

	Three Months Ended		Year Ending
	March 31,	2015	December 31, 2016
	2016		Guidance
GAAP Net Loss	\$ (85.1)	\$ (67.5)	\$(355) to \$(385)
Interest expense, net	8.2	8.8	35.0
Benefit from income taxes	(4.0)	(7.2)	(20.0) - (57.0)
Depreciation expense	13.1	7.9	35.0 - 45.0
Amortization expense	7.5	2.6	45.0
Stock-based compensation expense	30.2	22.7	140.0 - 167.0
Contingent consideration expense ⁽¹⁾	2.9	0.3	45.0 - 50.0
Acquisition expenses ⁽²⁾	—	7.0	-
Non-GAAP Loss	\$ (27.2)	\$ (25.4)	\$(75) to \$(100)

(1) Represents the expense associated with the change in the fair value of contingent acquisition consideration payable for the period, resulting from changes in estimated probabilities and timing of achieving certain developmental milestones.

(2) The \$7.0 million in the three months ended March 31, 2015, represents transaction expenses for the acquisition of Prosensa Holding N.V.

The following reconciliation of the Company's GAAP Condensed Consolidated Statements of Operations to non-GAAP Net Loss provides the details of the effects of the non-GAAP adjustments on the components of the Company's operating results for each of the periods presented. Management believes that providing the effects of the non-GAAP adjustments on the components of operating results is useful to investors and, when reviewed in conjunction with the Company's GAAP results, provides additional information regarding key drivers of the Company's core operations. The Company uses operating results on both a GAAP and a non-GAAP basis internally for operating, budgeting and financial planning purposes.

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND RECONCILIATION OF non-GAAP ADJUSTMENTS

Three Months Ended March 31, 2016 and 2015

(In millions of U.S. dollars)

(Unaudited)

	Three Months Ended March 31, 2016			2015			Non-GAAP	
	GAAP	Non-GAAP Adjustments		GAAP	Non-GAAP Adjustments			
		Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments		GAAP	Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	
REVENUES:								
Net product revenues	\$ 235.4	\$ —	\$ —	\$ 235.4	\$ 201.3	\$ —	\$ —	\$ 201.3
Collaborative agreement revenues	0.2	—	—	0.2	0.4	—	—	0.4
Royalty, license and other revenues	1.1	—	—	1.1	1.2	—	—	1.2
Total revenues	236.7	—	—	236.7	202.9	—	—	202.9
OPERATING EXPENSES:								
Cost of sales	43.1	—	(1.6)	41.5	31.0	—	(1.3)	29.7
Research and development	158.8	(7.4)	(13.7)	137.7	142.1	(3.6)	(10.0)	128.5
Selling, general and administrative	105.3	(5.7)	(14.9)	84.7	92.8	(4.3)	(18.4)	70.1
Intangible asset amortization and contingent consideration	10.4	(7.5)	(2.9)	—	2.9	(2.6)	(0.3)	—
Total operating expenses	317.6	(20.6)	(33.1)	263.9	268.8	(10.5)	(30.0)	228.3
LOSS FROM OPERATIONS	(80.9)	20.6	33.1	(27.2)	(65.9)	10.5	30.0	(25.4)
Equity in the loss of								
BioMarin/Genzyme LLC	(0.1)	—	—	(0.1)	(0.2)	—	—	(0.2)
Interest income	1.6	(1.6)	—	—	0.7	(0.7)	—	—
Interest expense	(9.8)	9.8	—	—	(9.5)	9.5	—	—
Debt conversion expense	—	—	—	—	(0.2)	—	—	(0.2)
Other income	0.1	—	—	0.1	0.4	—	—	0.4
LOSS BEFORE INCOME TAXES	(89.1)	28.8	33.1	(27.2)	(74.7)	19.3	30.0	(25.4)
Benefit from income taxes	(4.0)	4.0	—	—	(7.2)	7.2	—	—

NET LOSS \$ (85.1) \$ 24.8 \$ 33.1 \$ (27.2) \$ (67.5) \$ 12.1 \$ 30.0 \$ (25.4)

The following summarizes the reconciliation of the components of the non-GAAP adjustments to Cost of sales, Research and development and Selling, general and administrative expenses from the GAAP to the non-GAAP presentation (in millions of U.S. dollars, unaudited):

	Three Months Ended December 31,	
	2015	2014
Cost of sales - GAAP	\$ 43.1	\$ 31.0
Less: non-GAAP adjustments:		
Stock-based compensation	(1.6)	(1.3)
Cost of sales - non-GAAP	\$ 41.5	\$ 29.7

	Three Months Ended December 31,	
	2015	2014
Research and development - GAAP	\$ 158.8	\$ 142.1
Less: non-GAAP adjustments:		
Stock-based compensation	(13.7)	(10.0)
Depreciation	(7.4)	(3.6)
Research and development - non-GAAP	\$ 137.7	\$ 128.5

	Three Months Ended December 31,	
	2015	2014
Selling, general and administrative - GAAP	\$ 105.3	\$ 92.8
Less: non-GAAP adjustments:		
Stock-based compensation	(14.9)	(11.4)
Depreciation	(5.7)	(4.3)
Acquisition expenses	—	(7.0)
Selling, general and administrative - non-GAAP	\$ 84.7	\$ 70.1

	Three Months Ended December 31,	
	2015	2014
Intangible asset amortization and contingent consideration - GAAP	\$ 10.4	\$ 2.9
Less: non-GAAP adjustments		
Intangible asset amortization	(7.5)	(2.6)
Contingent consideration	(2.9)	(0.3)
Intangible asset amortization and contingent consideration - non-GAAP	\$ —	\$ —

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