BioMarin InvestorRoom

BioMarin Announces Fourth Quarter and Full Year 2016 Financial Results

- Full Year 2016 Total BioMarin Revenues Increase 26% Year over Year to $1.12 billion
- Full Year 2017 Total BioMarin Revenue Guidance of Between $1.25 billion to $1.30 billion

SAN RAFAEL, Calif., Feb. 23, 2017 /PRNewswire/ --

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

	Three Months Ended December 31,			Twelve Months Ended December 31,		
	2016	2015	% Change	2016	2015	% Change
Total BioMarin Revenue	$ 300	$ 228	32%	$ 1,117	$ 890	26%
Aldurazyme Net Product Revenue	35	39	(10)%	94	98	(4)%
Kuvan Net Product Revenue	90	65	38%	348	239	46%
Naglazyme Net Product Revenue	75	60	25%	297	303	(2)%
Vimizim Net Product Revenue	94	59	59%	354	228	55%
GAAP Net Income (Loss)	$ (91)	$ 69		$ (630)	$ (172)	
GAAP Net Income (Loss) per Share - Basic	$ (0.53)	$ 0.43		$ (3.80)	$ (1.07)	
GAAP Net Income (Loss) per Share - Diluted	$ (0.53)	$ 0.39		$ (3.81)	$ (1.07)	
Non-GAAP Loss [(1)]	$ (27)	$ (70)		$ (36)	$ (143)	

	December 31, 2016	December 31, 2015
Cash, cash equivalents and investments	$ 1,398	$ 1,018

(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 9 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 fourth quarter and year ended December 31, 2016. For the quarter ended December 31, 2016, GAAP net loss was $91 million, or ($0.53) per basic and diluted share, compared to GAAP net income of $69 million, or $0.43 and $0.39 per basic and diluted share, respectively, for the fourth quarter of 2015. GAAP net loss for the year ended December 31, 2016 was $630 million, or ($3.80) and ($3.81) per basic and diluted share, respectively, compared to GAAP net loss of $172 million, or ($1.07) per basic and diluted share for the year ended December 31, 2015. The change in GAAP net loss year over year was primarily due to the impairment of intangible assets associated with the discontinuance of the Kyndrisa and reveglucosidase alfa programs in 2016, and the absence of the gain on the sale of rights to talazoparib in 2015.

Non-GAAP loss for the year ended December 31, 2016 was $36 million, compared to non-GAAP loss of $143 million for the year ended December 31, 2015. 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 9 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 $1.12 billion for the year ended December 31, 2016, an increase of 26% compared to the same period in 2015. For the fourth quarter of 2016, Total BioMarin Revenues were $300 million, an increase of 32% compared to the same period in 2015. Vimizim net product revenues increased to $94 million in the quarter, a 59% year over year increase, primarily driven by 40% growth in new patients on Vimizim therapy year over year. For the fourth quarter of 2016, Naglazyme net product revenues increased to $75 million, an increase of 25% year over year. Patients on Naglazyme therapy continue to show consistent growth with an increase of 9% year over year. For the fourth quarter of 2016, Kuvan net product revenues increased to $90 million, a 38% year over year increase, including $70 million contributed from revenue in North America. Growth was driven by a 15% increase in patients on Kuvan therapy in North America, and $20 million contributed from net product revenues in ex-North American territories newly acquired in 2016.

As of December 31, 2016, BioMarin had cash, cash equivalents and investments totaling $1.4 billion, which includes $713 million of net proceeds from the August 12, 2016 public offering, as compared to $1.0 billion on December 31, 2015.

Commenting on the year, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin said, "2016 was a transformational year for BioMarin. Our commercial business topped $1 billion for the first time while we concurrently achieved significant clinical and regulatory milestones across the product pipeline. In the third quarter, we shared proof-of-concept data with BMN 270 gene therapy, the only factor VIII product in clinical development for the treatment of severe Hemophilia A. In addition, our regulatory filings for approval of Brineura, for the treatment of Batten disease, were accepted and validated in both the U.S. and EU. With the Prescription Drug User Fee Act (PDUFA) goal date for an FDA approval decision of April 27, 2017, we hope to have an approved treatment option for this devastating childhood disease in the near future."

Mr. Bienaimé continued, "In addition, in 2016 we moved our vosoritide program forward based on Phase 2 results in children ages 5-14 with achondroplasia. At the end of 2016, we initiated a one-year, randomized, placebo-controlled Phase 3 study in children with achondroplasia ages 5-14 using a daily 15µg/kg dose. We recently provided encouraging preliminary results with our earliest clinical-stage program BMN 250 for the treatment of MPS IIIB, or Sanfilippo Syndrome, Type B. We are now moving to the expansion phase of the development program that will assess the impact of treatment with BMN 250 on the neurocognitive function in this rapidly progressive pediatric brain disease. Finally, in addition to the filing of the BLA for pegvaliase for the treatment of phenylketonuria expected in the second quarter of 2017, we expect to start to turn the corner to profitability with the achievement of positive non-GAAP results for the full year 2017."

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

Total BioMarin Revenues

	Three Months Ended December 31,				Twelve Months Ended December 31,			
	2016	**2015**	**$ Change**	**% Change**	**2016**	**2015**	**$ Change**	**% Change**
Aldurazyme	$ 35	$ 39	$ (4)	-10%	$ 94	$ 98	$ (4)	-4%
Kuvan [(1)]	90	65	25	38%	348	239	109	46%
Naglazyme [(2)]	75	60	15	25%	297	303	(6)	-2%
Vimizim [(2)]	94	59	35	59%	354	228	126	55%
Royalty and other revenues	2	2	—	0%	6	5	1	20%
Total BioMarin revenues	**$ 300**	**$ 228**	**$ 72**	**32%**	**$1,117**	**$ 890**	**$ 227**	**26%**

(1) For the twelve months ended December 31, 2016, Kuvan net product revenues from North America contributed $272 million with an additional $76 million coming from the newly acquired ex-North American territories.

(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 December 31,				Twelve Months Ended December 31,			
	2016	**2015**	**$ Change**	**% Change**	**2016**	**2015**	**$ Change**	**% Change**
Aldurazyme revenue reported by Genzyme	$ 54	$ 54	$ —	0%	$ 223	$ 218	$ 5	2%

	Three Months Ended December 31,			Twelve Months Ended December 31,		
	2016	**2015**	**$ Change**	**2016**	**2015**	**$ Change**
Revenues earned based on Genzyme net sales	$ 27	$ 27	$ —	$ 98	$ 96	$ 2
Net product transfer revenues[(3)]	$ 8	$ 12	$ (4)	$ (4)	$ 2	$ (6)
Total Aldurazyme net product revenues	$ 35	$ 39	$ (4)	$ 94	$ 98	$ (4)

(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

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

- **BMN 270 gene therapy product for hemophilia A:** Today the Company announced that in the ongoing Phase 1/2 study, the three additional patients to be enrolled in the study will be dosed at the same 4 x 10^{13} vg/kg dose as the 3 most recently enrolled patients. Consistent with the dosing regimen of the 3 most recently enrolled patients, the 3 additional patients will be dosed without prophylactic corticosteroids. In October 2016, the Medicines and Healthcare Products Regulatory Agency (MHRA) in the United Kingdom approved continued enrollment into the open-label Phase 1/2 study of BMN 270 for severe hemophilia A. The agency also approved the Company's proposed amendments to the study, which included eliminating the requirement for prophylactic corticosteroids and increasing potential additional enrollment from up to three additional patients to up to six additional patients. In the fourth quarter of 2016, three patients were dosed at 4 x 10^{13} vg/kg with BMN 270.

  On February 1, 2017, BioMarin Pharmaceutical Inc. announced that the European Medicines Agency (EMA) had granted access to its Priority Medicines (PRIME) regulatory initiative for the company's investigational gene therapy treatment for severe hemophilia A, BMN 270.

  To be accepted for PRIME, an investigational therapy has to show its potential to benefit patients with unmet medical needs based on early clinical data. BMN 270 gene therapy is the first and only product candidate for hemophilia A to have received this designation.

  On January 9, 2017, the Company announced an update to its positive interim results of an open-label Phase 1/2 study with BMN 270. These data were an update from previously reported results in July 2016. A total of nine patients with severe hemophilia A received a single dose of BMN 270, seven of whom have been treated at the highest dose of 6 x 10^{13} vg/kg.

  As of the December 9, 2016 data cutoff, post-treatment follow-up ranges from 34 to 50 weeks. Median Factor VIII levels for the high dose cohort have been consistently within the normal range from 20 weeks through 44 weeks of treatment. According to the World Federation of Hemophilia rankings of severity of hemophilia A, the normal range of Factor VIII activity levels is between 50%-150%, expressed as a percentage of normal factor activity in blood. For those seven patients, as of each patient's most recent reading, six of seven patients continue to have Factor VIII activity levels above 50% and the seventh continues to be above 15%, which is the mild range (5%-40%) of hemophilia A.

  For the six patients at the high dose and previously on a Factor VIII prophylactic regimen, the mean annualized bleeding rate dropped 91% from 16.3 before the BMN 270 infusion to 1.5 two weeks after being dosed (median annualized bleeding rate dropped from 16.5 to 0). For those same six patients, the mean annualized Factor VIII infusions fell 98% from 136.7 to 2.9 (median annualized Factor VIII infusions fell from 138.5 to 0).

  In addition, six of the seven patients at the high dose as of January 9, 2017 were within the normal alanine aminotransferase (ALT) range, and one patient was less than 5% above the upper limit of normal, which is 43 U/L for the central laboratory in this study. At that time all patients had successfully tapered off of steroids with no lasting significant impact on Factor VIII expression or ALT levels.

  The Company intends to provide the next program update by the start of 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.

- **Vosoritide for achondroplasia:** On December 12, 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 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 whose growth plates are still open. This is approximately 25 percent of people with achondroplasia.

  The primary endpoint of the study is the change in growth velocity from baseline over one year in children treated compared to placebo. 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.

- **Brineura for CLN2, late-infantile form of Batten disease:** During the third quarter of 2016, the Company announced that the U.S. Food and Drug Administration (FDA) had accepted for review the submission of a Biologics License Application (BLA) for Brineura, an investigational therapy to treat children with CLN2 disease, a form of Batten disease. The PDUFA goal date for a decision is April 27, 2017.

  The FDA granted Brineura Priority Review status, which is designated for drugs that offer major advances in treatment or provide a treatment where no adequate therapy exists. Brineura was previously granted Orphan Drug Designation and Breakthrough Therapy Designation by the FDA. BioMarin also received validation of the Marketing Authorization Application (MAA) to the EMA for Brineura. Assuming a positive opinion from the Committee for Medicinal Products for Human Use (CHMP) and standard assessment timing, a decision from the European Commission is anticipated by the third quarter of 2017. The EMA previously granted Brineura Orphan Drug Designation.

- **Pegvaliase for phenylketonuria (PKU):** In March 2016, 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. Based on the supportive data results, the Company plans to submit a BLA to the FDA in the second quarter of 2017.

- **BMN 250 for MPS IIIB (Sanfilippo Syndrome, Type B):** On January 9, 2017, 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 brain 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 looking at improvement 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 fourth quarter and full year 2016 financial results today, Thursday, February 23, 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: 60631310

Replay Dial-in Number: 855.859.2056
Replay International Dial-in Number: 404.537.3406
Conference ID: 60631310

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.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, Brineura, 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; 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 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 PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

December 31, 2016 and December 31, 2015

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

	December 31, 2016	December 31, 2015[(1)]
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 408,330	$ 397,040

Short-term investments	381,347	195,579
Accounts receivable, net (allowance for doubtful accounts: $73 and $93, at December 31, 2016 and 2015, respectively)	215,280	164,959
Inventory	355,126	271,683
Other current assets	61,708	60,378
Total current assets	1,421,791	1,089,639
Noncurrent assets:		
Long-term investments	572,711	425,652
Property, plant and equipment, net	798,768	704,207
Intangible assets, net	553,780	683,996
Goodwill	197,039	197,039
Deferred tax assets	446,786	220,191
Other assets	32,815	408,644
Total assets	$ 4,023,690	$ 3,729,368
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	370,505	392,511
Short-term convertible debt, net	22,478	—
Short-term contingent acquisition consideration payable	46,327	52,946
Total current liabilities	439,310	445,457
Noncurrent liabilities:		
Long-term convertible debt, net	660,761	662,286
Long-term contingent acquisition consideration payable	115,310	32,663
Long-term deferred tax liabilities	—	143,527
Other long-term liabilities	42,034	44,588
Total liabilities	1,257,415	1,328,521
Stockholders' equity:		
Common stock, $0.001 par value: 250,000,000 shares authorized at December 31, 2016 and 2015: 172,647,588 and 161,526,044 shares issued and outstanding at December 31, 2016 and 2015, respectively.	173	162
Additional paid-in capital	4,288,113	3,414,837
Company common stock held by Nonqualified Deferred Compensation Plan	(14,321)	(13,616)
Accumulated other comprehensive income	12,816	21,033
Accumulated deficit	(1,520,506)	(1,021,569)
Total stockholders' equity	2,766,275	2,400,847
Total liabilities and stockholders' equity	$ 4,023,690	$ 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.

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

Three and Twelve Months Ended December 31, 2016 and 2015

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

(Unaudited)

	Three Months Ended	Twelve Months Ended

	December 31,		December 31,	
	2016	2015	2016	2015
REVENUES:				
Net product revenues	$298,186	$226,420	$1,110,381	$ 884,522
Royalty, license and other revenues	1,905	1,516	6,473	5,373
Total revenues	300,091	227,936	1,116,854	889,895
OPERATING EXPENSES:				
Cost of sales	64,147	48,044	209,620	152,008
Research and development	175,242	176,118	661,905	634,806
Selling, general and administrative	142,958	113,907	476,593	402,271
Intangible asset amortization and contingent consideration	7,365	(40,654)	(26,953)	(17,690)
Impairment of intangible assets	—	198,700	599,118	198,700
Gain on sale of intangible asset	—	(369,498)	—	(369,498)
Total operating expenses	389,712	126,617	1,920,283	1,000,597
NET INCOME (LOSS) FROM OPERATIONS	(89,621)	101,319	(803,429)	(110,702)
Equity in the loss of BioMarin/Genzyme LLC	(164)	(278)	(538)	(817)
Interest income	2,926	1,451	7,487	4,501
Interest expense	(9,732)	(9,333)	(39,499)	(38,244)
Other income (expense)	4,425	(194)	4,929	(9,462)
NET INCOME (LOSS) BEFORE INCOME TAXES	(92,166)	92,965	(831,050)	(154,724)
Provision for (benefit from) income taxes	(1,446)	24,348	(200,840)	17,075
NET INCOME (LOSS)	$ (90,720)	$ 68,617	$ (630,210)	$(171,799)
NET INCOME (LOSS) PER SHARE, BASIC	$ (0.53)	$ 0.43	$ (3.80)	$ (1.07)
NET INCOME (LOSS) PER SHARE, DILUTIVE	$ (0.53)	$ 0.39	$ (3.81)	$ (1.07)
Weighted average common shares outstanding, basic	172,006	161,149	165,985	160,025
Weighted average common shares outstanding, diluted	172,240	174,288	166,219	160,025

Non-GAAP Information

The results presented in this press release for the three and twelve months ended December 31, 2016 and 2015 as well as 2017 full-year guidance 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 Income (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 December 31,		Twelve Months Ended December 31,		Year Ending December 31, 2017
	2016	2015	2016	2015	Guidance
GAAP Net Loss	**$ (91.0)**	**$ 69.0**	**$ (630.0)**	**$ (172.0)**	**$(180) - $(140)**
Interest expense, net	7.0	8.0	32.0	34.0	35
Provision for (benefit from) income taxes	(1.0)	24.0	(201.0)	17.0	(60) - (100)
Depreciation expense	14.0	10.0	56.0	35.0	45 - 55
Amortization expense	7.0	3.0	30.0	11.0	30
Stock-based compensation expense	37.0	30.0	135.0	111.0	135 - 155
Contingent consideration expense [1]	—	(44.0)	(57.0)	(29.0)	25 - 35
Acquisition expenses[2]	—	—	—	7.0	-
Impairment charges[3]	—	199.0	599.0	212.0	-
Gain on sale of intangible asset[4]	—	(369.0)	—	(369.0)	-
non-GAAP Income (Loss)	$ (27.0)	$ (70.0)	$ (36.0)	$ (143.0)	$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 and Twelve Months Ended December 31, 2016 and 2015

(In millions of U.S. dollars)

(Unaudited)

Three Months Ended December 31,

	2016				2015			
		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-Based Compensation, Contingent Consideration and Other Adjustments	non-GAAP
Cost of sales	**$ 64.0**	$ —	$ (3.0)	$ 61.0	**$ 48.0**	$ —	$ (2.0)	$ 46.0
Research and development	**175.0**	(8.0)	(15.0)	152.0	**176.0**	(4.0)	(14.0)	158.0
Selling, general and administrative	**143.0**	(6.0)	(19.0)	118.0	**114.0**	(6.0)	(14.0)	94.0
Intangible asset amortization and contingent consideration [1]	**7.0**	(7.0)	—	—	**(41.0)**	(3.0)	44.0	—
Impairment of intangible assets[3]	—	—	—	—	**199.0**	—	(199.0)	—
Gain on sale of intangible asset [4]	—	—	—	—	**(369.0)**	—	369.0	—
Interest expense, net	**(7.0)**	7.0	—	—	**(8.0)**	8.0	—	—
Other income (expense)	**4.0**	—	—	4.0	**—**	—	—	—
Provision for								

(benefit from) income taxes	**(1.0)**	1.0	—	—	**24.0**	(24.0)	—	—
Net Income (Loss)/non-GAAP Loss	**(91.0)**	27.0	37.0	(27.0)	**69.0**	45.0	(184.0)	(70.0)

	Twelve Months Ended December 31,							
	2016				**2015**			
		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-Based Compensation, Contingent Consideration and Other Adjustments	non-GAAP
Cost of sales	**$ 210.0**	$ —	$ (9.0)	$ 201.0	**$ 152.0**	$ —	$ (7.0)	$ 145.0
Research and development	**662.0**	(31.0)	(58.0)	573.0	**635.0**	(16.0)	(49.0)	570.0
Selling, general and administrative[2]	**477.0**	(25.0)	(68.0)	384.0	**402.0**	(19.0)	(62.0)	321.0
Intangible asset amortization and contingent consideration [1]	**(27.0)**	(30.0)	57.0	—	**(18.0)**	(11.0)	29.0	—
Impairment of intangible assets[3]	**599.0**	—	(599.0)	—	**199.0**	—	(199.0)	—
Gain on sale of intangible asset [4]	**—**	—	—	—	**(369.0)**	—	369.0	—
Interest expense, net	**(32.0)**	32.0	—	—	**(34.0)**	34.0	—	—
Other income (expense)	**(4.0)**	—	—	(4.0)	**(10.0)**	—	13.0	3.0
Provision for (benefit from) income taxes	**(201.0)**	201.0	—	—	**17.0**	(17.0)	—	—
Net Loss/non-GAAP Loss	**(630.0)**	(83.0)	677.0	(36.0)	**(172.0)**	97.0	(68.0)	(143.0)

(1) Includes 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 regulatory and commercial milestones. Amounts for the year ended December 31, 2016 include $44 million and $21 million related to the change in probability of achieving the Kyndrisa and reveglucosidase alfa development milestones, respectively, as a result of discontinuance of these programs in June 2016.

(2) Includes $7 million of acquisition costs for the year ended December 31, 2015 related to the acquisition of Prosensa Holdings N.V.

(3) Includes $574 million and $25 million for the impairment of intangible assets associated with the discontinuance of the Kyndrisa and reveglucosidase alfa development programs, respectively, in June 2016. The $199 million in the three and twelve months ended December 31, 2015, primarily represents the impairment of a portion of the Kyndrisa in-process research and development (IPR&D) intangible assets.

(4) The $369 million gain in the three and twelve months ended December 31, 2015, represents the net gain on the sale of the Company's world-wide rights to talazoparib to Medivation Inc.

Contact:

Investors:
Traci McCarty
BioMarin Pharmaceutical Inc.
(415) 455-7558

Media:
Debra Charlesworth
BioMarin Pharmaceutical Inc.
(415) 455-7451

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