BioMarin Announces Third Quarter 2017 Financial Results

- Company Announces Total Revenues of $334.1 million in the Third Quarter of 2017; Full-year Revenue Guidance Confirmed at between $1.29 billion and $1.32 billion
- GAAP Net Loss Guidance Reduced to between $110 million and $130 million for Full-year 2017
- Non-GAAP Income Guidance Increased to between $60 million and $80 million for Full-year 2017

SAN RAFAEL, Calif., Oct. 26, 2017 /PRNewswire/ --

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2017	2016	% Change	2017	2016	% Change
Total Revenues	$ 334.1	$ 279.9	19 %	$ 955.3	$ 816.8	17 %
Aldurazyme Net Product Revenues	22.4	23.7	(5) %	61.7	58.8	5 %
Brineura Net Product Revenues	3.1	—	n/a	3.4	—	n/a
Kuvan Net Product Revenues	105.8	90.9	16 %	300.1	257.8	16 %
Naglazyme Net Product Revenues	72.1	77.8	(7) %	238.4	221.6	8 %
Vimizim Net Product Revenues	90.3	80.9	12 %	299.3	260.3	15 %
GAAP Net Loss	$ (12.5)	$ (37.4)		$ (65.7)	$ (539.5)	
GAAP Net Loss per Share - Basic	$ (0.07)	$ (0.22)		$ (0.38)	$ (3.29)	
GAAP Net Loss per Share - Diluted	$ (0.07)	$ (0.22)		$ (0.38)	$ (3.30)	
Non-GAAP Income (Loss) (1)	$ 7.8	$ 2.9		$ 68.7	$ (8.9)	

	September 30, 2017	December 31, 2016
Cash, cash equivalents and investments	$ 1,673.4	$ 1,362.4

(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, in certain periods, 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 third quarter ended September 30, 2017. For the quarter ended September 30, 2017, GAAP Net Loss was $(12.5) million, or $(0.07) per basic and diluted share, compared to GAAP Net Loss of $(37.4) million, or $(0.22) per basic and diluted share for the quarter ended September 30, 2016. The reduction in GAAP Net Loss year over year was primarily due to the $31.5 million net upfront license payment received as a result of the License and Settlement Agreements entered into with Sarepta Therapeutics Inc. in July 2017. The decreased GAAP Net Loss was also driven by increased net product revenues for Kuvan and Vimizim, partially offset by a decrease in the Benefit From Income Taxes, and increased Selling, General and Administrative expenses for Kuvan, Brineura and Vimizim. BioMarin also announced today that full year GAAP net loss guidance is being reduced to between ($110) million and ($130) million.

Non-GAAP Income for the third quarter ended September 30, 2017 was $7.8 million, compared to Non-GAAP Income of $2.9 million for the quarter ended September 30, 2016. BioMarin also announced today that full year Non-GAAP Income guidance is being increased to between $60 million and $80 million.

Total Revenues were $334.1 million for the third quarter of 2017, and were $955.3 million for the nine months ended September 30, 2017, an increase of 19% and 17% respectively compared to the same periods in 2016. For the nine months ended September 30, 2017, Kuvan net product revenues increased 16% year over year. Growth was driven by a 9% increase in the number of commercial patients on Kuvan therapy in the U.S and the continued growth in the ex-North American territories acquired in 2016. For the nine months ended September 30, 2017, Naglazyme net product revenues increased by 8% year over year, due primarily to an increase of 7% in the number of Naglazyme commercial patients. Vimizim net product revenues increased 15% year over year during the nine months ended September 30, 2017. The number of Vimizim commercial patients increased 23% year over year.

On October 18, 2017, the Company commented on its Total Revenue and Non-GAAP Income (Loss) trends for the third quarter and full-year 2017. In terms of the overall commercial business, BioMarin stated that sales of products in markets throughout most of the world are performing at or above internal expectations. However, the Company said the one exception is Brazil, where a slowdown in federal purchasing orders had extended into the third quarter of this year. As a result, third quarter revenues were negatively impacted. Since October 18, the Brazilian Ministry of Health has initiated their purchasing process which is expected to result in net product revenue from Brazil in the fourth quarter. Based on this order Total Revenues for full-year 2017 are confirmed to be within prior guidance.

As of September 30, 2017, BioMarin had cash, cash equivalents and investments totaling approximately $1.7 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 achieved a number of important strategic milestones so far this year, including record Total Revenues in the third quarter and the go ahead from both U.S. and U.K. health authorities to begin Phase 3 studies with valoctocogene roxaparvovec (formerly referred to as BMN 270) gene therapy program for severe hemophilia A by year-end." Mr. Bienaimé continued, "We had many significant updates at our recent R&D Day, including the announcement of our next IND candidate BMN 290 for Freidriech's Ataxia, a rare neurologic disorder that affects nearly 15,000 people worldwide. We were also pleased to share that vosoritide for achondroplasia demonstrated a sustained increase in annualized growth rate at 30 months of treatment. For pegvaliase, we anticipate FDA action on our Biologics License Application in the first half of 2018, as well as our planned submission of the Marketing Authorization Application in Europe in the first quarter of 2018. With these programs all advancing, supported by our strong base commercial business, we have reduced our GAAP Net Loss guidance and increased our Non-GAAP Income guidance for the full-year 2017."

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

	Three Months Ended September 30,				Nine Months Ended September 30,			
	2017	2016	$ Change	% Change	2017	2016	$ Change	% Change
Aldurazyme	$ 22.4	$ 23.7	$ (1.3)	(5) %	$ 61.7	$ 58.8	$ 2.9	5 %
Brineura	3.1	—	3.1	n/a	3.4	—	3.4	n/a
Firdapse	5.1	5.0	0.1	2 %	14.0	13.7	0.3	2 %
Kuvan (1)	105.8	90.9	14.9	16 %	300.1	257.8	42.3	16 %

Naglazyme [2]	72.1	77.8	(5.7)	(7) %	238.4	221.6	16.8	8 %
Vimizim [2]	90.3	80.9	9.4	12 %	299.3	260.3	39.0	15 %
Net Product Revenues	298.8	278.3	20.5	7 %	916.9	812.2	104.7	13 %
Royalty and Other Revenues	35.3	1.6	33.7		38.4	4.6	33.8	
Total Revenues	$ 334.1	$ 279.9	$ 54.2	19 %	$ 955.3	$ 816.8	$ 138.5	17 %

(1) Kuvan revenue growth was driven by a 9% increase in the number of commercial patients on Kuvan therapy in the U.S. and continued growth in 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 September 30,				Nine Months Ended September 30,			
	2017	2016	$ Change	% Change	2017	2016	$ Change	% Change
Aldurazyme revenue reported by Genzyme	$ 58.4	$ 58.9	$ (0.5)	(1) %	$ 176.3	$ 168.5	$ 7.8	5 %

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2017	2016	$ Change	2017	2016	$ Change
Royalties earned from Genzyme	$ 24.5	$ 26.9	$ (2.4)	$ 74.2	$ 71.2	$ 3.0
Net product transfer revenues [3]	(2.1)	(3.2)	1.1	(12.5)	(12.4)	(0.1)
Total Aldurazyme net product revenues	$ 22.4	$ 23.7	$ (1.3)	$ 61.7	$ 58.8	$ 2.9

(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

Full-year Revenue Guidance ($ in millions)

Item

	Provided August 2, 2017	Updated October 26, 2017
Total Revenues	$1,285 to $1,335	$1,290 to $1,320
Kuvan Net Product Revenues	$380 to $410	$400 to $420
Naglazyme Net Product Revenues	$300 to $330	$310 to $330
Vimizim Net Product Revenues	$400 to $430	$400 to $420

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

Item

	Provided August 2, 2017	Updated October 26, 2017
Cost of Sales (% of Total Revenues)	17.5% to 18.5%	17.5% to 18.5%
Research and Development Expense	$610 to $640	$600 to $620
Selling, General and Admin. Expense	$530 to $560	$530 to $550
GAAP Net Loss	$(115) to $(155)	$(110) to $(130)
Non-GAAP Income	$30 to $70	$60 to $80

Key Program Updates at R&D Day October 18, 2017

- **BMN 290 for Freidriech's Ataxia (FA):** BioMarin announced that it has selected as its next drug development candidate, BMN 290, a selective chromatin modulation therapy intended for treatment of FA. FA is a rare autosomal recessive disorder with worldwide prevalence of approximately 15,000, which results in disabling neurologic and cardiac progressive decline. Currently there are no approved disease modifying therapies for FA. In preclinical models, BMN 290 increases frataxin expression in affected tissues more than two-fold. BMN 290 is a second-generation compound derived from a compound the Company acquired from Repligen Corporation (Repligen) that had human clinical data demonstrating increases in frataxin in FA patients. The Company selected BMN 290 for its favorable penetration into the central nervous system and cardiac target tissues, and its preservation of the selectivity of the original Repligen compound. The Company expects to submit the IND application for BMN 290 in the second half of 2018.

- **Valoctocogene roxaparvovec (formerly referred to as BMN 270) gene therapy for hemophilia A:** BioMarin announced today that it had been granted Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA) for valoctocogene roxaparvovec. The designation is intended to expedite the development and review of medicines to treat a serious disease and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies. The Company also announced that the 6e13 vg/kg dose and 4e13 vg/kg dose had been cleared by both U.S. and U.K. health authorities to begin Phase 3 studies.

  The protocol for each Phase 3 study, one using the 6e13 vg/kg dose and one using the 4e13 vg/kg dose, will likely include approximately 40 patients for a duration of 52 weeks per study. The Company expects to file for approval of valoctocogene roxaparvovec with 52-week data from the Phase 3 studies. BioMarin expects to initiate the global Phase 3 program in the fourth quarter of 2017, complete enrollment of the last patient by the end of 2018 and provide top-line Phase 3 data by the end of 2019.

  At R&D Day, the Company provided an update on the ongoing open-label Phase 1/2 study of the 4e13 vg/kg dose at up to 36 weeks of observation at the September 14, 2017 data cut. Since the last data update provided during the second quarter earnings call on August 2, 2017, five of the six patients at the 4e13 vg/kg dose tracked to the low range of normal, and the sixth is in the mild range for Factor VIII levels. Median annualized bleed and factor VIII use rates for 4e13 and 6e13 vg/kg were zero after Week 4.

  The World Health Organization (WHO) has approved, and BioMarin was issued, the International Nonproprietary Name (INN) "valoctocogene roxaparvovec" for the Company's gene therapy to treat hemophilia A. INNs identify pharmaceutical substances or active pharmaceutical ingredients. Each INN is a unique name that is globally recognized and is public property. A nonproprietary name is also known as a generic name.

BioMarin has commissioned its gene therapy manufacturing facility, located in Novato, California. Good Manufacturing Practices (GMP) production of valoctocogene roxaparvovec has commenced and is intended to support clinical development activities and anticipated commercial demand, upon product approval. 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.

- **Pegvaliase for phenylketonuria (PKU):** BioMarin announced that the pegvaliase Biologics License Application (BLA) remains on track for FDA action during the first half of 2018. The Company plans to submit a Marketing Authorization Application to the European Medicines Agency in the first quarter of 2018. Pegvaliase is a PEGylated recombinant phenylalanine ammonia lyase enzyme product that reduces blood phenylalanine (Phe) levels in adult patients with PKU who have uncontrolled blood Phe levels on existing management.

- **Vosoritide for achondroplasia:** BioMarin provided an update on its open-label Phase 2 study of vosoritide, an analog of C-type Natriuretic Peptide (CNP), in children with achondroplasia, the most common form of disproportionate short stature in humans.

  Vosoritide for achondroplasia has demonstrated sustained increase in average growth velocity over 30 months of treatment in 10 children, who completed 30 months of daily dosing at 15 µg/kg/day. Over this period of time, patients experienced mean absolute growth increase of approximately 4 cm over what their baseline growth velocity would have predicted.

  The sustained increase in annualized growth velocity was accompanied by sustained improvements over time in height compared to age- and gender-matched unaffected children as measure by z-scores. In addition, treatment with vosoritide shows continued improvement over time in proportionality as measured by a ratio of the upper and lower body measurements, or U/L ratio.

  The ongoing, global 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. The Company expects to complete enrollment of the Phase 3 study in mid-2018 and provide top-line data in the second half of 2019.

  Given the importance of early intervention in this indication, at R&D Day, the Company announced that it will begin an infant/toddler study in the first half of 2018 in children ages 0-5 years old.

- **BMN 250 for MPS IIIB (Sanfilippo Syndrome, Type B):** The Company discussed preliminary results from the Phase 1/2 trial with BMN 250 that demonstrated reduced heparan sulfate (HS) levels, a biomarker in the cerebrospinal fluid (CSF), in the brains of affected children. BMN 250, is an investigational enzyme replacement therapy using a novel fusion of recombinant human alpha-N-acetylglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of Sanfilippo B syndrome or mucopolysaccharidosis IIIB (MPS IIIB). Discovered by BioMarin, BMN 250 is being studied in a multicenter, international clinical trial evaluating safety and tolerability, as well as cognitive function of patients with Sanfilippo B receiving BMN 250. Designed to restore functional NAGLU activity in the brain, BMN 250 is administered via intracerebroventricular (ICV) infusion.

  In the completed dose escalation portion of the study (Part 1), which was primarily designed to determine safety and pharmacodynamic activity of BMN 250, three patients received escalating doses (30mg, 100mg, 300mg) of BMN 250 over 9 to12 months. CSF HS levels, which were markedly elevated at baseline, were reduced to the non-affected or normal range in all three patients, whether assessed as total or disease-specific HS. Sanfilippo B patients are missing one of four enzymes for HS degradation.

  In those same patients, abdominal MRI scans showed significantly enlarged liver size at baseline followed by rapid decreases in liver size into the normal range for age with BMN 250 treatment, suggesting that ICV-administered BMN 250 reaches the peripheral circulation and may have activity in somatic organs. In contrast, most Sanfilippo B patients enrolled in BioMarin's concurrently-running observational study (250-901) had increased liver size at baseline and experienced further increases in liver size over time. Two of the three treated patients from the dose escalation arm showed stabilization or some improvement compared to their pre-dose baselines in cognitive Development Quotient (DQ), a measure of cognitive function normalized to age. Patients with untreated Sanfilippo B usually show progressive decline in DQ.

Conference Call Details

BioMarin will host a conference call and webcast to discuss third quarter 2017 financial results today, Thursday, October 26, 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: 574.990.1362
Conference ID: 96054850

Replay Dial-in Number: 855.859.2056
Replay International Dial-in Number: 404.537.3406
Conference ID: 96054850

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 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 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, including BioMarin's Phase 3 program with valoctocogene roxaparvovec; the ongoing Phase 3 study of vosoritide and the Phase 1/2 study of BMN 250; the continued clinical development and commercialization of BioMarin's commercial products and product candidates; including the filing of an IND for BMN 290 in the second half of 2018; the possible approval and commercialization of BioMarin's product candidates, including the filing of a Marketing Authorization Application for pegvaliase in Europe in the first quarter of 2018; the adequacy of production of valoctocogene roxaparvovec in the Company's commercial gene therapy manufacturing facility; and actions by regulatory authorities, including the expected FDA action on the pegvaliase BLA during the first half of 2018. 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 June 30, 2017 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
September 30, 2017 and December 31, 2016
(In thousands of U.S. dollars, except share and per 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 172,647,588 shares issued and outstanding as of September 30, 2017 and December 31, 2016, respectively.	176	173
Additional paid-in capital	4,435,449	4,288,113
Company common stock held by Nonqualified Deferred Compensation Plan	(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 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 and Nine Months Ended September 30, 2017 and 2016
(In thousands of U.S. dollars, except per share amounts)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
REVENUES:				
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 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)
NET LOSS PER SHARE, DILUTED	$ (0.07)	$ (0.22)	$ (0.38)	$ (3.30)
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

Non-GAAP Information

The results presented in this press release for the three and nine months ended September 30, 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 Royalty and Other Revenues, 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 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 Loss to Non-GAAP Income (Loss)
(In millions of U.S. dollars)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,		Year Ending December 31, 2017
	2017	2016	2017	2016	Guidance
GAAP Net Loss	**$ (12.5)**	**$ (37.4)**	**$ (65.7)**	**$ (539.5)**	**$(130) - $(110)**
Interest expense, net	6.9	8.3	21.0	25.2	30
Benefit from income taxes	(8.1)	(29.4)	(24.8)	(199.4)	(20) - (50)
Depreciation expense	13.3	18.8	36.9	42.7	45 - 55
Amortization expense	7.6	7.5	22.7	22.6	30
Stock-based compensation expense	35.9	32.9	106.7	97.3	130 - 150
Contingent consideration expense [1]	(3.8)	2.2	3.4	(56.9)	10
Impairment charges [2]	—	—	—	599.1	-
Royalty and other revenues [3]	(31.5)	—	(31.5)	—	(35)
Non-GAAP Income (Loss)	$ 7.8	$ 2.9	$ 68.7	$ (8.9)	$60 - $80

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
(In millions of U.S. dollars)
(Unaudited)

	Three Months Ended September 30,							
	2017				2016			
		Adjustments				Adjustments		
	GAAP Reported	Interest, Taxes, Depreciation and Amortization	Royalty and Other Revenues, Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non-GAAP	GAAP Reported	Interest, Taxes, Depreciation and Amortization	Royalty and Other Revenues, Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non-GAAP
Royalty and other revenues [3]	**$ 35.4**	$ —	$ (31.5)	$ 3.9	**$ 1.6**	$ —	$ —	$ 1.6
Cost of sales	**59.5**	—	(3.0)	56.5	**50.7**	—	(2.1)	48.6
Research and development	**154.1**	(7.6)	(13.8)	132.7	**160.8**	(11.1)	(14.2)	135.5
Selling, general and administrative	**130.5**	(5.7)	(19.1)	105.7	**118.8**	(7.7)	(16.6)	94.5
Intangible asset amortization and contingent consideration [1]	**3.8**	(7.6)	3.8	—	**9.7**	(7.5)	(2.2)	—
Impairment of intangible assets [2]	**—**	—	—	—	**—**	—	—	—
Interest expense, net	**(6.9)**	6.9	—	—	**(8.3)**	8.3	—	—
Benefit from income taxes	**(8.1)**	8.1	—	—	**(29.4)**	29.4	—	—

GAAP Net Loss/Non-GAAP Income (Loss)	**(12.5)**	19.7	0.6	7.8	**(37.4)**	5.2	35.1	2.9

	Nine Months Ended September 30,							
	2017				2016			
		Adjustments				Adjustments		
	GAAP Reported	Interest, Taxes, Depreciation and Amortization	Royalty and Other Revenues, Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non-GAAP	**GAAP Reported**	Interest, Taxes, Depreciation and Amortization	Royalty and Other Revenues, Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non-GAAP
Royalty and other revenues [3]	**$ 38.5**	$ —	$ (31.5)	$ 7.0	**$ 4.6**	$ —	$ —	$ 4.6
Cost of sales	**165.8**	—	(7.8)	158.0	**145.5**	—	(6.0)	139.5
Research and development	**442.1**	(20.2)	(40.0)	381.9	**486.7**	(23.4)	(43.0)	420.3
Selling, general and administrative	**394.1**	(16.7)	(58.9)	318.5	**333.6**	(19.3)	(48.3)	266.0
Intangible asset amortization and contingent consideration [1]	**26.1**	(22.7)	(3.4)	—	**(34.3)**	(22.6)	56.9	—
Impairment of intangible assets [2]	**—**	—	—	—	**599.1**	—	(599.1)	—
Interest expense, net	**(21.0)**	21.0	—	—	**(25.2)**	25.2	—	—
Benefit from income taxes	**(24.8)**	24.8	—	—	**(199.4)**	199.4	—	—
GAAP Net Loss/Non-GAAP Income (Loss)	**(65.7)**	55.8	78.6	68.7	**(539.5)**	(108.9)	639.5	(8.9)

1. Amounts for the three and nine months ended September 30, 2016 include $43.8 million and $21.1 million related to the change in probability of achieving the Kyndrisa and Reveglucosidase alfa development milestones, respectively.
2. Amounts or the three and nine months ended September 30, 2016 include $574.1 million and $25.0 million for the impairment of intangible assets associated with the discontinuance of the Kyndrisa and Reveglucosidase alpha development programs, respectively.
3. Primarily represents the one-time upfront payment related to the License and Settlement Agreement entered into with Sarepta Therapeutics, Inc. in July 2017.

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