

BioMarin Announces Fourth Quarter and Full Year 2017 Financial Results

- Record Total Revenues of \$1.3 Billion for Full Year 2017 Representing an 18% Increase Year over Year
- Full Year 2017 GAAP Net Loss Decreased to \$117 Million and Non-GAAP Income Increased to \$74 Million

SAN RAFAEL, Calif., Feb. 22, 2018 /PRNewswire/ --

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

| | Three Months Ended December 31, | | | Twelve Months Ended December 31, | | |
|--|------------------------------------|---------------------|----------|-------------------------------------|------------|----------|
| | 2017 | 2016 | % Change | 2017 | 2016 | % Change |
| Total Revenues | \$ 358.3 | \$ 300.1 | 19% | \$ 1,313.6 | \$ 1,116.9 | 18% |
| Aldurazyme Net Product Revenues | 28.3 | 35.0 | (19)% | 90.0 | 93.8 | (4)% |
| Brineura Net Product Revenues | 5.2 | — | n/a | 8.6 | — | n/a |
| Kuvan Net Product Revenues | 107.4 | 90.2 | 19% | 407.5 | 348.0 | 17% |
| Naglazyme Net Product Revenues | 93.8 | 74.9 | 25% | 332.2 | 296.5 | 12% |
| Vimizim Net Product Revenues | 114.0 | 93.8 | 22% | 413.3 | 354.1 | 17% |
| GAAP Net Loss | \$ (51.4) | \$ (90.7) | | \$ (117.0) | \$ (630.2) | |
| GAAP Net Loss per Share - Basic | (0.29) | (0.53) | | (0.67) | (3.80) | |
| GAAP Net Loss per Share - Diluted | (0.30) | (0.53) | | (0.67) | (3.81) | |
| Non-GAAP Income (Loss) ⁽¹⁾ | \$ 5.2 | \$ (27.5) | | \$ 74.0 | \$ (36.5) | |
| | December 31, | December 31, | | | | |
| | 2017 | 2016 | | | | |
| Cash, cash equivalents and investments | \$ 1,781.7 | \$ 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 10 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, 2017. For the quarter ended December 31, 2017 GAAP Net Loss was \$51.4 million, or \$0.29 and \$0.30 per basic and diluted share, respectively, compared to GAAP Net Loss of \$90.7 million, or \$0.53 per basic and diluted share, respectively, for the quarter ended December 31, 2016. GAAP Net Loss for the year ended December 31, 2017 was \$117.0 million, or \$0.67 per basic and diluted share, respectively, compared to GAAP Net Loss of \$630.2 million, or \$3.80 and \$3.81 per basic and diluted share, respectively, for the year ended December 31, 2016. The reduction in GAAP Net Loss year over year was primarily due to increased net product revenues for Kuvan, Naglazyme and Vimizim, the \$31.5 million net upfront license payment received from Sarepta Therapeutics Inc. (Sarepta) in connection with the settlement of the Company's patent proceedings against Sarepta, the \$125.0 million gain on sale of intangible assets related to the sale of the Priority Review Voucher, and the absence of the impairment of intangible assets associated with the discontinuance of the Kyndrisa and reveglucosidase alfa programs in 2016, partially offset by an increase in the provision for income taxes related to the impact of the Tax Cuts and Jobs Act on the Company's operating results.

Non-GAAP Income for the three months ended December 31, 2017 was \$5.2 million, compared to Non-GAAP Loss of \$27.5 million for the three months ended December 31, 2016. Non-GAAP Income for the year ended December 31, 2017 was \$74.0 million, compared to Non-GAAP Loss of \$36.5 million for the year ended December 31, 2016.

Total Revenues were \$1.3 billion for the year ended December 31, 2017 compared to \$1.1 billion for the year ended December 31, 2016, an increase of 18%. For the year ended December 31, 2017, Kuvan net product revenues increased 17% year over year. Growth was driven by a 7% increase in the number of commercial patients on Kuvan therapy in North America resulting in 21% revenue growth in that region. For the year ended December 31, 2017, Vimizim net product revenues increased by 17% year over year, due primarily to an increase of 20% in the number of Vimizim commercial patients. Naglazyme net product revenues increased 12% year over year during the year ended December 31, 2017. The number of Naglazyme commercial patients increased 6% year over year.

As of December 31, 2017, BioMarin had cash, cash equivalents and investments totaling approximately \$1.8 billion, as compared to \$1.4 billion on December 31, 2016.

Commenting on the year and the quarter, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "2017 was a momentous year for BioMarin driven by numerous financial, regulatory and clinical achievements. We reduced our GAAP Net Loss and achieved our goal of Non-GAAP profitability for the full year supported by continued strong demand for our commercial products. On the regulatory front, during the first half of 2017, we received U.S. and EU approval of our sixth commercial product, Brineura, the first treatment approved for CLN2 disease. Our late-stage clinical programs all advanced as expected, including vosoritide, pegvaliase and valoctocogene roxaparvovec (formerly referred to as BMN 270). In addition to receiving PRIME and Breakthrough Therapy designations for valoctocogene roxaparvovec gene therapy for severe hemophilia A, we shared new 1.5 year results demonstrating sustained FVIII levels within the normal range. This achievement led to the start of our global GENER8-1 and GENER8-2 Phase 3 studies for the treatment of patients with severe hemophilia A."

Mr. Bienaimé continued, "With pegvaliase, we were pleased to have submitted our Biologics License Application in June, which was filed for review by the FDA and received a Priority Review designation. We anticipate FDA action on our Biologics License Application for pegvaliase at the end of May 2018. In addition, we plan to submit our Marketing Authorization Application for pegvaliase in Europe in the first quarter of 2018. Finally, we shared many significant updates at our R&D Day in the fourth quarter, including the announcement of our next IND candidate BMN 290 for Friedreich'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. We begin 2018 poised to move our 5 potential new product candidates to their next stage of clinical or regulatory development while driving top line revenues from our six commercialized products."

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

Total Revenues

| | Three Months Ended December 31, | | | | Twelve Months Ended December 31, | | | |
|--|------------------------------------|------|-----------|----------|-------------------------------------|------|-----------|----------|
| | 2017 | 2016 | \$ Change | % Change | 2017 | 2016 | \$ Change | % Change |



| | | | | | | | | |
|----------------------------|-----------------|-----------------|----------------|-------|-------------------|-------------------|-----------------|------|
| Aldurazyme | \$ 28.3 | \$ 35.0 | \$ (6.7) | (19)% | \$ 90.0 | \$ 93.8 | \$ (3.8) | (4)% |
| Brineura | 5.2 | — | 5.2 | n/a | 8.6 | — | 8.6 | n/a |
| Firdapse | 4.8 | 4.3 | 0.5 | 12% | 18.8 | 18.0 | 0.8 | 4% |
| Kuvan ⁽¹⁾ | 107.4 | 90.2 | 17.2 | 19% | 407.5 | 348.0 | 59.5 | 17% |
| Naglazyme ⁽²⁾ | 93.8 | 74.9 | 18.9 | 25% | 332.2 | 296.5 | 35.7 | 12% |
| Vimizim ⁽²⁾ | 114.0 | 93.8 | 20.2 | 22% | 413.3 | 354.1 | 59.2 | 17% |
| Net Product Revenues | <u>353.5</u> | <u>298.2</u> | <u>55.3</u> | 19% | <u>1,270.4</u> | <u>1,110.4</u> | <u>160.0</u> | 14% |
| Royalty and other revenues | 4.8 | 1.9 | 2.9 | | 43.2 | 6.5 | 36.7 | |
| Total revenues | <u>\$ 358.3</u> | <u>\$ 300.1</u> | <u>\$ 58.2</u> | 19% | <u>\$ 1,313.6</u> | <u>\$ 1,116.9</u> | <u>\$ 196.7</u> | 18% |

(1) Kuvan revenue growth was driven by a 7% increase in the number of commercial patients on Kuvan therapy in the U.S.

(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, | | | |
|--|--|----------------|-----------------|----------|-------------------------------------|----------------|-----------------|----------|
| | 2017 | 2016 | \$ Change | % Change | 2017 | 2016 | \$ Change | % Change |
| | Aldurazyme revenue reported by Genzyme | \$ 57.5 | \$ 54.8 | \$ 2.7 | 5 % | \$ 233.8 | \$ 223.3 | \$ 10.5 |
| Revenues earned based on Genzyme net sales | \$ 27.9 | \$ 26.9 | \$ 1.0 | | \$ 102.1 | \$ 98.1 | \$ 4.0 | |
| Net product transfer revenues ⁽³⁾ | 0.4 | 8.1 | (7.7) | | (12.1) | (4.3) | (7.8) | |
| Total Aldurazyme net product revenues | <u>\$ 28.3</u> | <u>\$ 35.0</u> | <u>\$ (6.7)</u> | | <u>\$ 90.0</u> | <u>\$ 93.8</u> | <u>\$ (3.8)</u> | |

(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.

2018 Financial Guidance

Full-year Revenue Guidance (\$ in millions)

Item

| | 2018 Guidance |
|--------------------------------|--------------------|
| Total Revenues | \$1,470 to \$1,530 |
| Kuvan Net Product Revenues | \$440 to \$480 |
| Naglazyme Net Product Revenues | \$325 to \$355 |
| Vimizim Net Product Revenues | \$460 to \$500 |
| Brineura Net Product Revenues | \$35 to \$55 |

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

Item

| | 2018 Guidance |
|-------------------------------------|--------------------|
| Cost of Sales (% of Total Revenues) | 20.0% to 21.0% |
| Research and Development Expense | \$645 to \$685 |
| Selling, General and Admin. Expense | \$575 to \$615 |
| GAAP Net Loss | \$(115) to \$(165) |
| Non-GAAP Income | \$100 to \$140 |

*All Financial Guidance items are calculated based on Generally Accepted Accounting Principles (GAAP) with the exception of Non-GAAP Income. Refer to Non-GAAP Information beginning on page 10 of this press release for a complete discussion of the Company's Non-GAAP financial information and reconciliations to the comparable GAAP reported information.

Key Program Highlights

- Valoctogene roxaparveoc (formerly referred to as BMN 270) gene therapy for hemophilia A:** In December at the 2017 American Society of Hemophilia (ASH) meeting, the Company provided new 1.5 year results demonstrating the 6e13 vg/kg dose of valoctogene roxaparveoc achieved sustained factor VIII levels within the normal range in severe hemophilia A for most patients. The data presented at ASH had a cut off of November 16, 2017 and included a number of updates. For the 6e13 vg/kg dose, at 78 weeks post infusion, the median and mean factor VIII levels for patients were 90 and 89%, respectively. Median annualized bleed and factor VIII use rates for the 6e13 vg/kg cohort were zero after week 4. Mean annualized bleed and factor VIII use rates for the 6e13 vg/kg cohort were 0.5 and 6.1, respectively. For the 4e13 vg/kg dose, the three patients with the longest follow-up (at week 48) had factor VIII activity levels that were in or near the normal range with both median and mean values of 49%. Median annualized bleed and factor VIII use rates for the 4e13 vg/kg cohort were zero after week 4 and when their factor VIII activity rose above 5%. Mean annualized bleed and factor VIII use rates for the 4e13vg/kg cohort were 0.6 and 2.0, respectively.

Also at ASH, the Company announced that the New England Journal of Medicine (NEJM) published an independent, peer-reviewed article on the ongoing Phase 1/2 study

of valoctocogene roxaparvovec in men with severe hemophilia A. The article assessed the safety and efficacy of valoctocogene roxaparvovec at the 6e13 dose, after 52 weeks. The NEJM article, "AAV Gene Transfer in Patients with Severe Hemophilia A," reported sustained normalization of factor VIII activity over the 52-week period for six of seven study participants who received the 6e13 vg/kg dose of valoctocogene roxaparvovec. In addition, the article stated that all seven participants demonstrated stabilization of hemostasis and a profound reduction in factor VIII use.

In December, the Company announced that it had dosed the first patient in the global GENE8-1 Phase 3 study with the 6e13 vg/kg dose of valoctocogene roxaparvovec for the treatment of patients with severe hemophilia A.

The global Phase 3 program includes two studies with valoctocogene roxaparvovec, one with the 6e13 vg/kg dose (GENE8-1) and one with the 4e13 vg/kg dose (GENE8-2). Both Phase 3 GENE8 studies will be open-label single-arm studies to evaluate the efficacy and safety of valoctocogene roxaparvovec. The Company expects to enroll the first patient in the GENE8-2 study in early 2018. The primary endpoint in both studies will be based on the factor VIII activity level achieved following valoctocogene roxaparvovec, and the secondary endpoints will measure annualized factor VIII replacement therapy use rate and annualized bleed rate.

BioMarin also plans to begin a Phase 1/2 Study with the 6e13kg/vg dose and with approximately 10 patients who are AAV5 positive. The first patient is expected to enroll in the first half of 2018.

- **Pegvaliase for phenylketonuria (PKU):** In the second quarter of 2017, BioMarin announced that the pegvaliase Biologics License Application (BLA) had been filed and it remains on track for U.S. Food and Drug Administration (FDA) action in May of 2018. In the third quarter of 2017, the Company received Priority Review designation for pegvaliase. BioMarin plans to submit a Marketing Authorization Application (MAA) to the European Medicines Agency in the first quarter of 2018. Pegvaliase is a PEGylated recombinant phenylalanine ammonialyase enzyme product to reduce blood phenylalanine (Phe) levels in adult patients with PKU who have uncontrolled blood Phe levels on existing management.
- **Vosoritide for achondroplasia:** In the fourth quarter of 2017, BioMarin provided an update of 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 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 Company's multi-pronged program was developed to demonstrate the ability to improve clinical outcomes in children with achondroplasia. The program includes four distinct areas of focus to support global approval. Currently enrolling, the 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. The feeder study in the U.S. is fully enrolled and the Company expects to complete enrollment of the Phase 3 study in mid-2018. BioMarin expects to provide top-line data in the second half of 2019.

The long-term, open-label Phase 2 program to corroborate maintenance of effect is anticipated to provide over 5 years of clinical data at the time of the planned New Drug Application submission. Given the importance of early intervention in this indication, the Company intends to begin an infant/toddler study in 2018 in children 0-5 years old. Finally, the Company has undertaken a Natural History program to augment clinical understanding of outcomes of untreated patients for comparison to treated patients.

- **BMN 250 for MPS IIIB (Sanfilippo Syndrome, Type B):** On February 7, 2018 at the WORLD Symposium 2018, the Company updated preliminary results from the Phase 1/2 trial with BMN 250, 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). In 6 of 6 BMN 250 treated subjects, normalization of heparan sulfate (HS) levels, a biomarker in the cerebrospinal fluid (CSF), was observed. Normalization of liver size in 3 of 3 BMN 250 treated subjects was also observed. These data suggest that BMN 250, which is administered via intracerebroventricular (ICV) infusion, reaches peripheral circulation and has activity in somatic organs. Development Quotient (DQ), a measure of cognitive function normalized to age, was also observed. In 3 of 3 treated patients from the dose escalation arm of the study, preliminary data suggest stabilization of cognitive DQ at the high dose of BMN 250 in all subjects. Patients with untreated Sanfilippo B syndrome usually show progressive decline in DQ.

Invented 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, the same delivery modality used to treat children with Brineura.

- **BMN 290 for Friedreich's Ataxia:** In the fourth quarter of 2017, BioMarin announced that it had selected as its next drug development candidate, BMN 290, a selective chromatin modulation therapy intended for treatment of Friedreich's ataxia. Friedreich's ataxia is a rare autosomal recessive disorder that results in disabling neurologic and cardiac progressive decline. Prior to the compound being acquired by BioMarin from Repligen Corporation (Repligen), it demonstrated increases in frataxin in Friedreich's ataxia patients. In preclinical models, BMN 290 increases frataxin expression in brain tissues more than two-fold. 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. Currently, there are no approved disease modifying therapies for Friedreich's ataxia. The Company expects to submit the IND application for BMN 290 in the second half of 2018.
- **BioMarin sells second priority review voucher for \$125.0 million:** In December 2017, the Company sold the Rare Pediatric Disease Priority Review Voucher (PRV) it obtained in April of 2017 for a lump sum payment of \$125.0 million. The Company received the voucher under a FDA program intended to encourage the development of treatments for rare pediatric diseases. BioMarin was awarded the voucher when it received approval of Brineura®, a new biological product for patients with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency, a form of Batten disease.
- **The impact of the 2017 Tax Act on the Company's 2017 provision for income taxes:** On December 22, 2017, the bill known as the Tax Cuts and Jobs Act (the 2017 Tax Act) was signed into law, resulting in significant changes to the U.S. corporate income tax system. These changes include a federal statutory rate reduction from 35% to 21% and the elimination or reduction of certain domestic deductions and credits, including a 50% reduction in the orphan drug credit benefit. The 2017 Tax Act changed U.S. international taxation from a worldwide basis to a modified territorial system that includes base erosion prevention measures on foreign earnings, which will result in the Company's foreign subsidiaries being subject to U.S. taxation in the future. These changes are effective in 2018.

The provision for income taxes was \$81.2 million for the year ended December 31, 2017 compared to a benefit from income taxes of \$200.8 million for the year ended December 31, 2016. Changes to tax laws and tax rates are required to be accounted for in the period of the enactment, therefore the income tax provision for the year ended December 31, 2017 included the impact of the 2017 Tax Act. The provision for (benefit from) income taxes for the year ended December 31, 2017 included a provisional expense of \$42.3 million related to the 2017 Tax Act, primarily consisting of \$33.1 million for the re-measurement of the net deferred tax assets at the lower enacted corporate tax rate and \$9.2 million related to the new limitations on tax deductible compensation. The Company's deferred tax assets and liabilities have been measured at the enacted tax rate expected to apply when these temporary differences are expected to be realized or settled. Additionally, the Company also assessed the impact of the 2017 Tax Act on the Company's financial projections and concluded that it is more likely than not that these state tax credits will not be utilized in the foreseeable future, and recognized \$41.1 million of income tax expense during the fourth quarter of 2017 to establish a valuation allowance against those state tax credits because these credits do not expire and the Company projects that it will be generating more credits than it will utilize on an annual basis. The 2017 Tax Act also includes a one-time mandatory deemed repatriation toll tax on accumulated earnings of the Company's foreign subsidiaries that did not impact the Company, due to a net deficit in these foreign subsidiaries.

Conference Call Details

BioMarin will host a conference call and webcast to discuss third quarter 2017 financial results today, Thursday, February 22, 2018 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 | Replay Dial-in Number: 855.859.2056 |
| International Dial-in Number: 574.990.1362 | Replay International Dial-in Number: 404.537.3406 |

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.biomin.com.

Forward-Looking Statement

This press release and the associated conference call and webcast contain 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 and Phase 1/2 study with valoctocogene roxaparvec; the ongoing Phase 2 and Phase 3 studies 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 roxaparvec in the Company's commercial gene therapy manufacturing facility; actions by regulatory authorities, including the expected FDA action on the pegvaliase BLA at the end of May 2018 and the effect of the Tax Cuts and Jobs Act. 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, 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[®], Brineura[®], Firdapse[®], Kuvan[®], Naglazyme[®] 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**

December 31, 2017 and December 31, 2016

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

| | December 31, 2017 | December 31, 2016(1) |
|--|------------------------------|---------------------------------|
| | (unaudited) | |
| ASSETS | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 598,028 | \$ 408,330 |
| Short-term investments | 797,940 | 381,347 |
| Accounts receivable, net | 261,365 | 215,280 |
| Inventory | 475,775 | 355,126 |
| Other current assets | 74,036 | 61,708 |
| Total current assets | <u>2,207,144</u> | <u>1,421,791</u> |
| Noncurrent assets: | | |
| Long-term investments | 385,785 | 572,711 |
| Property, plant and equipment, net | 896,700 | 798,768 |
| Intangible assets, net | 517,510 | 553,780 |
| Goodwill | 197,039 | 197,039 |
| Deferred tax assets | 399,095 | 446,786 |
| Other assets | 29,852 | 32,815 |
| Total assets | <u>\$ 4,633,125</u> | <u>\$ 4,023,690</u> |
| LIABILITIES AND STOCKHOLDERS' EQUITY | | |
| Current liabilities: | | |
| Accounts payable and accrued liabilities | \$ 401,921 | \$ 370,505 |
| Short-term convertible debt, net | 360,949 | 22,478 |
| Short-term contingent acquisition consideration payable | 53,648 | 46,327 |
| Total current liabilities | <u>816,518</u> | <u>439,310</u> |
| Noncurrent liabilities: | | |
| Long-term convertible debt, net | 813,521 | 660,761 |
| Long-term contingent acquisition consideration payable | 135,318 | 115,310 |
| Other long-term liabilities | 59,105 | 42,034 |
| Total liabilities | <u>1,824,462</u> | <u>1,257,415</u> |
| Stockholders' equity: | | |
| Common stock, \$0.001 par value: 500,000,000 and 250,000,000 shares authorized, respectively; 175,843,749 and 172,647,588 shares issued and outstanding, respectively. | 176 | 173 |
| Additional paid-in capital | 4,483,220 | 4,288,113 |
| Company common stock held by Nonqualified Deferred Compensation Plan (the NQDC) | (14,224) | (14,321) |
| Accumulated other comprehensive income (loss) | (22,961) | 12,816 |
| Accumulated deficit | (1,637,548) | (1,520,506) |
| Total stockholders' equity | <u>2,808,663</u> | <u>2,766,275</u> |
| Total liabilities and stockholders' equity | <u>\$ 4,633,125</u> | <u>\$ 4,023,690</u> |

- (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 Twelve Months Ended December 31, 2017 and 2016

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

(Unaudited)

| | Three Months Ended | | Twelve Months Ended | |
|--|--------------------|-------------|---------------------|--------------|
| | December 31, | | December 31, | |
| | 2017 | 2016 | 2017 | 2016 |
| REVENUES: | | | | |
| Net product revenues | \$ 353,577 | \$ 298,186 | \$ 1,270,445 | \$ 1,110,381 |
| Royalty and other revenues | 4,728 | 1,905 | 43,201 | 6,473 |
| Total revenues | 358,305 | 300,091 | 1,313,646 | 1,116,854 |
| OPERATING EXPENSES: | | | | |
| Cost of sales | 75,995 | 64,147 | 241,786 | 209,620 |
| Research and development | 168,608 | 175,242 | 610,753 | 661,905 |
| Selling, general and administrative | 160,280 | 142,958 | 554,336 | 476,593 |
| Intangible asset amortization and contingent consideration | 20,375 | 7,365 | 46,471 | (26,953) |
| Impairment of intangible assets | — | — | — | 599,118 |
| Gain on sale of intangible asset | (125,000) | — | (125,000) | — |
| Total operating expenses | 300,258 | 389,712 | 1,328,346 | 1,920,283 |
| INCOME (LOSS) FROM OPERATIONS | 58,047 | (89,621) | (14,700) | (803,429) |
| Equity in the loss of BioMarin/Genzyme LLC | (295) | (164) | (1,291) | (538) |
| Interest income | 4,822 | 2,926 | 14,853 | 7,487 |
| Interest expense | (11,664) | (9,732) | (42,707) | (39,499) |
| Other income (expense) | 3,688 | 4,425 | 7,970 | 4,929 |
| INCOME (LOSS) BEFORE INCOME TAXES | 54,598 | (92,166) | (35,875) | (831,050) |
| Provision for (benefit from) income taxes | 105,990 | (1,446) | 81,167 | (200,840) |
| NET LOSS | \$ (51,392) | \$ (90,720) | \$ (117,042) | \$ (630,210) |
| NET LOSS PER SHARE, BASIC | \$ (0.29) | \$ (0.53) | \$ (0.67) | \$ (3.80) |
| NET LOSS PER SHARE, DILUTED | \$ (0.30) | \$ (0.53) | \$ (0.67) | \$ (3.81) |
| Weighted average common shares outstanding, basic | 175,485 | 172,006 | 174,427 | 165,985 |
| Weighted average common shares outstanding, diluted | 175,705 | 172,240 | 174,427 | 166,219 |

Non-GAAP Information

The results presented in this press release for the three and twelve months ended December 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 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, Non-GAAP Gain on Sale of Intangible Assets, 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 | | Twelve Months Ended | | Year Ending December 31, 2018 Guidance |
|---|--------------------|-----------|---------------------|------------|--|
| | December 31, | | December 31, | | |
| | 2017 | 2016 | 2017 | 2016 | |
| GAAP Net Loss | \$ (51.4) | \$ (90.7) | \$ (117.0) | \$ (630.2) | \$ (115.0) - \$(165.0) |
| Interest expense, net | 6.8 | 6.8 | 27.9 | 32.0 | 25.0 - 35.0 |
| Provision for (benefit from) income taxes | 106.0 | (1.4) | 81.2 | (200.8) | (40.0) - 0.0 |
| Depreciation expense | 14.8 | 13.1 | 51.7 | 55.8 | 50.0 - 60.0 |
| Amortization expense | 13.4 | 7.6 | 36.2 | 30.2 | 30.0 |
| Stock-based compensation expense | 33.6 | 37.3 | 140.2 | 134.6 | 150.0 - 170.0 |

| | | | | | | | | |
|--|---------|--------|--------|------|---------|--------|---------|--------|
| asset amortization and contingent consideration (1) | 46.5 | (36.2) | (10.3) | — | (27.0) | (30.2) | 57.2 | — |
| Impairment of intangible assets (2) | — | — | — | — | 599.1 | — | (599.1) | — |
| Gain on sale of intangible asset (3) | (125.0) | — | 125.0 | — | — | — | — | — |
| Interest expense, net | (27.9) | 27.9 | — | — | (32.0) | 32.0 | — | — |
| Provision for (benefit from) income taxes | 81.2 | (81.2) | — | — | (200.8) | 200.8 | — | — |
| GAAP Net Loss/Non- GAAP Income (Loss) | (117.0) | 197.0 | (6.0) | 74.0 | (630.2) | (82.8) | 676.5 | (36.5) |

1. Amounts include 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 \$43.8 million and \$21.1 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. Amounts for the twelve months ended December 31, 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. Amount represents gain on the sale of the Priority Review Voucher in December 2017.
4. 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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<http://investors.biopharm.com/2018-02-22-BioMarin-Announces-Fourth-Quarter-and-Full-Year-2017-Financial-Results>