

BioMarin Announces Second Quarter 2017 Financial Results

- Company Announces Record Total BioMarin Revenues of \$317.4 million in the Second Quarter of 2017

- GAAP Net Loss Reduced to \$36.8 million; Non-GAAP Income of \$26.6 million Reported in the Second Quarter of 2017

SAN RAFAEL, Calif., Aug. 2, 2017 /PRNewswire/ --

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



| | Three Months Ended June 30, | | | Six Months Ended June 30, | | |
|---|-----------------------------|--------------------------|----------|---------------------------|------------|----------|
| | 2017 | 2016 | % Change | 2017 | 2016 | % Change |
| Total BioMarin Revenues | \$ 317.4 | \$ 300.1 | 6 % | \$ 621.2 | \$ 536.9 | 16 % |
| Aldurazyme Net Product Revenues | 19.9 | 18.7 | 6 % | 39.3 | 35.1 | 12 % |
| Brineura Net Product Revenues | 0.3 | — | n/a | 0.3 | — | n/a |
| Kuvan Net Product Revenues | 102.0 | 90.2 | 13 % | 194.3 | 166.9 | 16 % |
| Naglazyme Net Product Revenues | 85.7 | 78.4 | 9 % | 166.3 | 143.8 | 16 % |
| Vimizim Net Product Revenues | 103.2 | 106.8 | (3) % | 209.0 | 179.4 | 16 % |
| GAAP Net Loss | \$ (36.8) | \$ (419.0) | | \$ (53.1) | \$ (502.1) | |
| GAAP Net Loss per Share - Basic and Diluted | \$ (0.21) | \$ (2.58) | | \$ (0.31) | \$ (3.10) | |
| Non-GAAP Income (Loss) ⁽¹⁾ | \$ 26.6 | \$ 17.0 | | \$ 61.0 | \$ (11.7) | |
| | June 30, 2017 | December 31, 2016 | | | | |
| Cash, cash equivalents and investments | \$ 1,209.8 | \$ 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 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 second quarter ended June 30, 2017. For the quarter ended June 30, 2017, GAAP net loss was \$36.8 million, or \$0.21 per basic and diluted share, compared to GAAP net loss of \$419.0 million, or \$2.58 per basic and diluted share for the quarter ended June 30, 2016. The reduction in GAAP net loss year over year was primarily due to the absence of the \$599.1 million impairment of intangible assets associated with the discontinuance of the Kyndrisa and Reveglucosidase alfa programs in 2016, partially offset by increased selling, general and administrative expenses for the continued global expansion of Kuvan and the launch of Brineura.

Non-GAAP income for the second quarter ended June 30, 2017 was \$26.6 million, compared to non-GAAP income of \$17.0 million for the quarter ended June 30, 2016. Non-GAAP income (loss) is defined by the Company as reported GAAP net income (loss), excluding net interest expense, provision for (benefit from) income taxes, depreciation expense, amortization expense, stock-based compensation expense, contingent consideration expense and certain other specified items as detailed below. Refer to non-GAAP Information beginning on page 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 \$317.4 million for the second quarter of 2017, an increase of 6% compared to the same period in 2016. For the second quarter of 2017, Kuvan net product revenues increased to \$102.0 million, a 13% year over year increase. Growth was driven by an 11% increase in the number of patients on Kuvan therapy in the U.S and the continued growth in the ex-North American territories acquired in 2016. For the second quarter of 2017, Naglazyme net product revenues increased to \$85.7 million, an increase of 9% year over year. The number of patients on Naglazyme therapy increased 7% year over year. Vimizim net product revenues were \$103.2 million in the quarter, a 3% year over year decrease, primarily driven by the timing of government orders. The number of patients on Vimizim therapy increased 24% year over year. With these new patient starts, Vimizim remains on track to meet full year revenue target of \$400 to \$430 million, a growth rate of 13% to 21%.

As of June 30, 2017, BioMarin had cash, cash equivalents and investments totaling \$1.2 billion, as compared to \$1.4 billion on December 31, 2016.

Commenting on the quarter, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "We achieved a number of important strategic milestones during the first half of 2017, including record revenues, the development and approval of Brineura in the U.S. and Europe in less than four years and unprecedented data with BMN 270, the first-in-human gene therapy product for the treatment of severe hemophilia A." Mr. Bienaimé continued, "We expect that trajectory to continue during the second half of 2017 as we execute our Phase 3 program developing both the 4e13 vg/kg and 6e13 vg/kg doses in two separate studies with BMN 270; filing the MAA for pegvaliase in Europe; and providing updates across our development pipeline at R&D Day on October 18. With these investments in our pipeline and commercial organization, while we expect to incur a GAAP net loss for 2017, we expect to achieve non-GAAP income for the full-year 2017, as planned."

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

Total BioMarin Revenues

Three Months Ended June 30,

Six Months Ended June 30,

| | 2017 | 2016 | \$ Change | % Change | 2017 | 2016 | \$ Change | % Change |
|----------------------------|-----------------|-----------------|----------------|----------|-----------------|-----------------|----------------|----------|
| Aldurazyme | \$ 19.9 | \$ 18.7 | 1.2 | 6 % | \$ 39.3 | \$ 35.1 | \$ 4.2 | 12 % |
| Brineura | 0.3 | — | 0.3 | n/a | 0.3 | — | 0.3 | n/a |
| Firdapse | 4.8 | 4.5 | 0.3 | 7 % | 8.9 | 8.7 | 0.2 | 2 % |
| Kuvan ⁽¹⁾ | 102.0 | 90.2 | 11.8 | 13 % | 194.3 | 166.9 | 27.4 | 16 % |
| Naglazyme ⁽²⁾ | 85.7 | 78.4 | 7.3 | 9 % | 166.3 | 143.8 | 22.5 | 16 % |
| Vimizim ⁽²⁾ | 103.2 | 106.8 | (3.6) | (3) % | 209.0 | 179.4 | 29.6 | 16 % |
| Net product revenues | <u>315.9</u> | <u>298.6</u> | <u>17.3</u> | 6 % | <u>618.1</u> | <u>533.9</u> | <u>84.2</u> | 16 % |
| Royalty and other revenues | 1.5 | 1.5 | — | | 3.1 | 3.0 | 0.1 | |
| Total BioMarin Revenues | <u>\$ 317.4</u> | <u>\$ 300.1</u> | <u>\$ 17.3</u> | 6 % | <u>\$ 621.2</u> | <u>\$ 536.9</u> | <u>\$ 84.3</u> | 16 % |

(1) Kuvan revenue growth was driven by an 11% increase in the number of 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 June 30, | | | | Six Months Ended June 30, | | | |
|--|-----------------------------|---------|-----------|----------|---------------------------|----------|-----------|----------|
| | 2017 | 2016 | \$ Change | % Change | 2017 | 2016 | \$ Change | % Change |
| Aldurazyme revenue reported by Genzyme | \$ 62.4 | \$ 56.8 | \$ 5.6 | 10 % | \$ 117.9 | \$ 109.6 | \$ 8.3 | 8 % |

| | Three Months Ended June 30, | | | Six Months Ended June 30, | | |
|--|-----------------------------|----------------|---------------|---------------------------|----------------|---------------|
| | 2017 | 2016 | \$ Change | 2017 | 2016 | \$ Change |
| Royalties earned from Genzyme | \$ 24.7 | \$ 22.8 | \$ 1.9 | \$ 49.6 | \$ 44.3 | \$ 5.3 |
| Net product transfer revenues ⁽³⁾ | (4.8) | (4.1) | (0.7) | (10.3) | (9.2) | (1.1) |
| Total Aldurazyme net product revenues | <u>\$ 19.9</u> | <u>\$ 18.7</u> | <u>\$ 1.2</u> | <u>\$ 39.3</u> | <u>\$ 35.1</u> | <u>\$ 4.2</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.

Updated 2017 Financial Guidance

Revenue Guidance (\$ in millions)

Item

| | <u>Provided May 4, 2017</u> | <u>Updated August 2, 2017</u> |
|--------------------------------|-----------------------------|-------------------------------|
| Total BioMarin Revenues | \$1,250 to \$1,300 | \$1,285 to \$1,335 |
| Kuvan Net Product Revenues | \$380 to \$410 | Unchanged |
| Naglazyme Net Product Revenues | \$300 to \$330 | Unchanged |
| Vimizim Net Product Revenues | \$400 to \$430 | Unchanged |

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

Item

| | <u>Provided May 4, 2017</u> | <u>Updated August 2, 2017</u> |
|--|-----------------------------|-------------------------------|
| Cost of Sales (% of Total BioMarin Revenues) | 17.5% to 18.5% | Unchanged |
| Research and Development Expense | \$620 to \$650 | \$610 to \$640 |
| Selling, General and Admin. Expense | \$520 to \$550 | \$530 to \$560 |
| GAAP Net Loss | \$(140) to \$(180) | \$(115) to \$(155) |
| Non-GAAP Income | \$30 to \$70 | Unchanged |

Key Program Updates

- **BMN 270 gene therapy for hemophilia A:** The Company announced today that it will expand its development plan for BMN 270, its investigational gene therapy for Hemophilia A, to include an additional Phase 3 study of the 4e13 vg/kg dose based on updated data as of July 28, 2017 from its ongoing open-label save Phase 1/2 study of BMN 270. Since the last data update presented at the International Society on Thrombosis and Haemostasis (ISTH) 2017 Congress, the Factor VIII activity levels in the 4e13 vg/kg cohort have continued to trend upwards and now support an additional Phase 3 study to the development program.

A total of six patients received a single dose of BMN 270 at the 4e13 vg/kg dose. Based on the most recent data, for the three patients who were given the 4e13 vg/kg dose in November/December 2016, at week 32, all are in or near to the normal range of Factor VIII activity levels, with both median and mean Factor VIII levels of 51%. For the cohort of three patients who were given the 4e13 vg/kg dose in February/March 2017, at week 20, their Factor VIII activity levels have all moved into the mild range and two of the three are continuing to trend upward. For all six patients who received a dose of 4e13 vg/kg, at week 20, the median Factor VIII level was 34% and the mean was 31%. According to the World Federation of Hemophilia rankings of severity of hemophilia A, the mild hemophilia A range of Factor VIII activity levels is between 5% and 40%, and the normal range of Factor VIII activity levels for people without disease is between 50% and 150%, in each case expressed as a percentage of normal factor activity in blood.

Based on these updated results, BioMarin plans to initiate two separate Phase 3 studies as soon as possible, one with the 4e13 vg/kg dose and one with the 6e13 vg/kg dose. In addition, the Company has commissioned its commercial gene therapy manufacturing facility and expects to start the Phase 3 program in the fourth quarter of 2017. (See press release on 4e13 dose update from earlier today)

6e13 vg/kg data as announced at ISTH July 11, 2017: As of the May 31, 2017 data cutoff as announced at ISTH, all patients at the 6e13 vg/kg dose had reached 52 weeks of post-treatment follow-up. Median and mean Factor VIII levels from week 20 through 52 for the 6e13 vg/kg dose cohort have been consistently within the normal levels post treatment as a percentage calculated based on the number of International Units per deciliter (IU/dL) of plasma. At one year after dosing, the median and mean Factor VIII levels of the 6e13 vg/kg cohort continue to be above 50%. For the six patients who were on pre-study prophylaxis after receiving a single dose of BMN 270 at 6e13 vg/kg dose and after reaching a Factor VIII level above 5%, the mean Annualized Bleed Rate (ABR) was reduced by 97% from 16.3 to 0.5. The median ABR for those same patients was reduced from 16.5 to zero. The mean annualized Factor VIII infusions for the 6e13 vg/kg dose cohort were reduced by 94% from 136.7 to 8.5. The median annualized Factor VIII infusions for those same patients were reduced from 138.5 to zero.

In the open-label Phase 1/2 study, a total of 15 patients with severe hemophilia A¹ (defined by the World Federation of Hemophilia (WFH) as having Factor VIII activity levels less than 1%, expressed as a percentage of normal factor activity in blood) received a single dose of BMN 270, seven of whom were treated at a dose of 6e13 vg/kg and an additional six of whom were subsequently treated at a lower dose of 4e13 vg/kg. The other two patients in the study were treated at lower doses as part of dose escalation in the study and did not achieve therapeutic efficacy.

- **Pegvaliase for phenylketonuria (PKU):** On June 30, 2017, the Company announced that it had submitted a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for pegvaliase, a PEGylated recombinant phenylalanine ammonia lyase enzyme product, to reduce blood phenylalanine (Phe) levels in adult patients with PKU who have uncontrolled blood Phe levels on existing management. Following receipt of the BLA, the FDA conducts an initial assessment of the application to determine whether to accept it for substantive review. We expect to hear from FDA within approximately two months of submission as to whether FDA is going to accept the application for substantive review. The Company also intends to submit an application for registration of pegvaliase in the European Union (EU) by year end 2017.

In 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.

- **Brineura for CLN2, late-infantile form of Batten disease:** On April 27, 2017, the Company announced that the U.S. Food and Drug Administration (FDA) had approved Brineura (cerliponase alfa), the first approved treatment for any form of Batten Disease.

On June 1, the Company announced that the European Commission (E.C.) had granted marketing authorization for Brineura, the first treatment approved in the European Union for the treatment of neuronal ceroid lipofuscinosis type 2 (CLN2), also known as tripeptidyl peptidase 1 (TPP1) deficiency. The dosing administration includes all ages from birth. With approval from the E.C., BioMarin received marketing authorization for Brineura in all 28 countries of the European Union, Norway, Iceland and Liechtenstein.

The commercial launch of Brineura is well underway in the U.S. and Europe. The Company recorded its first commercial sales of Brineura in the second quarter of 2017 in the U.S. and Argentina and in the third quarter of 2017, both France and Germany had their first commercial sales.

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

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

- **Vosoritide for achondroplasia:** In the second quarter of 2017, the Company continued enrollment in its global Phase 3 study for vosoritide, an analog of C-type Natriuretic Peptide (CNP), in children with achondroplasia, the most common form of dwarfism. The first child enrolled in the study was at a site in Australia. The primary endpoint of the study is the change in growth velocity from baseline over one year in children treated compared to placebo. Following discussions with global health authorities, the Company also plans to augment the growth velocity data in the Phase 3 study with assessments of proportionality, functionality and cumulative growth observed in that study and the ongoing Phase 2 study, as well as safety and efficacy in infants.

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

The Company intends to provide an update on the most recent data available with vosoritide at the upcoming R&D Day event in New York City on October 18, 2017.

- **BMN 250 for MPS IIIB (Sanfilippo Syndrome, Type B):** In the second quarter of 2017, the Company continued enrollment in the Phase 1/2 program with BMN 250 and announced preliminary results from the Phase 1/2 trial. From this trial, the Company observed BMN 250 reduced heparan sulfate levels, a biomarker in the cerebrospinal fluid (CSF), in the brains of affected children. Since sharing these results with 30mg/weekly dose, patients have been safely escalated to the highest dose of 300mg. The study has now moved to the expansion phase with the highest dose and measuring changes in neurocognitive function in children with this rapid and progressive neurodegenerative disease.
- **License agreement resolution subsequent to the second quarter end:** On July 18, 2017, the Company announced that it had executed a license agreement that provides Sarepta Therapeutics, Inc. (Sarepta) with global exclusive rights to BioMarin's Duchenne muscular dystrophy (DMD) patent estate for EXONDYS 51 and all future exon-skipping products. BioMarin retains the right to convert the license to a co-exclusive right in the event it decides to proceed with an exon-skipping therapy for DMD. In addition, Sarepta and BioMarin executed a settlement agreement, resolving the ongoing worldwide patent proceedings related to the use of EXONDYS 51 and all future exon-skipping products for the treatment of DMD.

Under the terms of the license and settlement agreements, Sarepta will make a one-time payment of \$35 million to BioMarin as well as certain additional regulatory and commercial milestone payments and net sales royalties for exons 51, 45, 53 and possibly on future exon-skipping products. The Company expects to recognize the \$35 million payment in the third quarter of 2017 as Royalty and other revenues and no amounts are expected to be deferred or amortized.

Conference Call Details

BioMarin will host a conference call and webcast to discuss second quarter 2017 financial results today, Wednesday, August 2, 2017 at 4:30 p.m. ET. This event can be accessed on the investor section of the BioMarin website at www.biomin.com.

U.S. / Canada Dial-in Number: 866.502.9859
International Dial-in Number: 547.990.1362
Conference ID: 41574149

Replay Dial-in Number: 855.859.2056
Replay International Dial-in Number: 404.537.3406
Conference ID: 41574149

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 contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about: the expectations of total BioMarin revenues, net product revenues and expenses for BioMarin's commercial products, GAAP net loss, non-GAAP income (loss) and other specified income statement guidance; the potential revenues and expenses related to BioMarin's product candidates, including BMN 270, pegvaliase, vosoritide and BMN 250; the financial performance of BioMarin as a whole; the timing of BioMarin's clinical studies and trials and announcements of data from those studies and trials, including BioMarin's registration-enabling Phase 3 program with BMN 270; the continued clinical development and commercialization of BioMarin's commercial products and product candidates; the possible approval and commercialization of BioMarin's product candidates, including the filing of a marketing authorization application for pegvaliase in Europe in the second quarter of 2017; the timing of the commencement of production of BMN 270 in the Company's commercial gene therapy manufacturing facility; and actions by regulatory authorities. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: BioMarin's success in the commercialization of its commercial products; Genzyme Corporation's success in continuing the commercialization of Aldurazyme; results and timing of current and planned preclinical studies and clinical trials, BioMarin's ability to successfully manufacture its commercial products and product candidates; the content and timing of decisions by the FDA, the European Commission and other regulatory authorities concerning each of the described products and product candidates; the market for each of these products; actual sales of BioMarin's commercial products; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission (SEC), including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's Quarterly Report on Form 10-Q for the quarter ended March 31, 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[®], Vimizim[®], Naglazyme[®], Kuvan[®] and Firdapse[®] are registered trademarks of BioMarin Pharmaceutical Inc., or its affiliates. Brineura[™] and Kyndrisa[™] are trademarks of BioMarin Pharmaceutical Inc. Aldurazyme[®] is a registered trademark of BioMarin/Genzyme LLC.

¹ Source: World Federation of Hemophilia
<http://www.wfh.org/en/resources/annual-global-survey>
<http://www.wfh.org/en/abd/prophylaxis/prophylaxis-administration-and-dosing-schedules>

CONDENSED CONSOLIDATED BALANCE SHEETS

June 30, 2017 and December 31, 2016

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

| | June 30, 2017 | December 31, 2016⁽¹⁾ |
|--|----------------------|--|
| | (unaudited) | |
| ASSETS | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 354,864 | \$ 408,330 |
| Short-term investments | 372,912 | 381,347 |
| Accounts receivable, net | 238,338 | 215,280 |
| Inventory | 429,831 | 355,126 |
| Other current assets | 62,875 | 61,708 |
| Total current assets | <u>1,458,820</u> | <u>1,421,791</u> |
| Noncurrent assets: | | |
| Long-term investments | 482,036 | 572,711 |
| Property, plant and equipment, net | 851,097 | 798,768 |
| Intangible assets, net | 538,565 | 553,780 |
| Goodwill | 197,039 | 197,039 |
| Deferred tax assets | 470,961 | 446,786 |
| Other assets | 21,447 | 32,815 |
| Total assets | <u>\$ 4,019,965</u> | <u>\$ 4,023,690</u> |
| LIABILITIES AND STOCKHOLDERS' EQUITY | | |
| Current liabilities: | | |
| Accounts payable and accrued liabilities | \$ 319,210 | \$ 370,505 |
| Short-term convertible debt, net | — | 22,478 |
| Short-term contingent acquisition consideration payable | 55,093 | 46,327 |
| Total current liabilities | <u>374,303</u> | <u>439,310</u> |
| Noncurrent liabilities: | | |
| Long-term convertible debt, net | 676,205 | 660,761 |
| Long-term contingent acquisition consideration payable | 122,899 | 115,310 |
| Other long-term liabilities | 50,979 | 42,034 |
| Total liabilities | <u>1,224,386</u> | <u>1,257,415</u> |
| Stockholders' equity: | | |
| Common stock, \$0.001 par value: 500,000,000 shares authorized; 175,248,847 and 172,647,588 shares issued and outstanding as of June 30, 2017 and December 31, 2016, respectively. | 175 | 173 |
| Additional paid-in capital | 4,397,980 | 4,288,113 |
| Company common stock held by Nonqualified Deferred Compensation Plan | (14,289) | (14,321) |
| Accumulated other comprehensive income (loss) | (14,658) | 12,816 |
| Accumulated deficit | (1,573,629) | (1,520,506) |
| Total stockholders' equity | <u>2,795,579</u> | <u>2,766,275</u> |
| Total liabilities and stockholders' equity | <u>\$ 4,019,965</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.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

Three and Six Months Ended June 30, 2017 and 2016

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

(Unaudited)

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|------------------------------------|---------------------|----------------------------------|---------------------|
| | 2017 | 2016 | 2017 | 2016 |
| REVENUES: | | | | |
| Net product revenues | \$ 315,926 | \$ 298,576 | \$ 618,116 | \$ 533,933 |
| Royalty and other revenues | 1,522 | 1,555 | 3,077 | 2,934 |
| Total revenues | 317,448 | 300,131 | 621,193 | 536,867 |
| OPERATING EXPENSES: | | | | |
| Cost of sales | 56,305 | 51,617 | 106,311 | 94,735 |
| Research and development | 143,039 | 167,039 | 288,042 | 325,832 |
| Selling, general and administrative | 143,505 | 109,577 | 263,524 | 214,877 |
| Intangible asset amortization and contingent consideration | 13,411 | (54,414) | 22,336 | (43,972) |
| Impairment of intangible assets | — | 599,118 | — | 599,118 |
| Total operating expenses | 356,260 | 872,937 | 680,213 | 1,190,590 |
| LOSS FROM OPERATIONS | (38,812) | (572,806) | (59,020) | (653,723) |
| Equity in the loss of BioMarin/Genzyme LLC | (220) | (135) | (743) | (270) |
| Interest income | 2,983 | 1,357 | 6,055 | 2,928 |
| Interest expense | (10,040) | (9,944) | (20,159) | (19,787) |
| Other income (expense) | 543 | (1,417) | 4,015 | (1,219) |
| LOSS BEFORE INCOME TAXES | (45,546) | (582,945) | (69,852) | (672,071) |
| Benefit from income taxes | (8,713) | (163,931) | (16,729) | (170,006) |
| NET LOSS | \$ (36,833) | \$ (419,014) | \$ (53,123) | \$ (502,065) |
| NET LOSS PER SHARE, BASIC AND DILUTED | \$ (0.21) | \$ (2.58) | \$ (0.31) | \$ (3.10) |
| Weighted average common shares outstanding, basic and diluted | 174,374 | 162,587 | 173,547 | 162,067 |

Non-GAAP Information

The results presented in this press release for the three and six months ended June 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, License 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)

| | | | | | | | | |
|----------------------------------|---------------|------|------|------|----------------|---------|---------|------|
| intangible assets ⁽²⁾ | — | — | — | — | 599.1 | — | (599.1) | — |
| Interest expense, net | (7.0) | 7.0 | — | — | (8.6) | 8.6 | — | — |
| Benefit from income taxes | (8.7) | 8.7 | — | — | (163.9) | 163.9 | — | — |
| GAAP Net Loss/non-GAAP Income | | | | | | | | |
| (Loss) | (36.8) | 17.6 | 45.8 | 26.6 | (419.0) | (135.3) | 571.3 | 17.0 |

Six Months Ended June 30,

| | 2017 | | | | 2016 | | | |
|---|----------------------|--------------------|--|----------|----------------------|--------------------|--|----------|
| | GAAP Reported | Adjustments | | | GAAP Reported | Adjustments | | |
| | | Amortization | Stock-Based Compensation, Contingent Consideration and Other Adjustments | non-GAAP | | Amortization | Stock-Based Compensation, Contingent Consideration and Other Adjustments | non-GAAP |
| Cost of sales | \$ 106.3 | \$ — | \$ (4.8) | \$ 101.5 | \$ 94.7 | \$ — | \$ (3.9) | \$ 90.8 |
| Research and development | 288.0 | (12.7) | (26.1) | 249.2 | 325.8 | (12.4) | (28.8) | 284.6 |
| Selling, general and administrative | 263.5 | (11.0) | (39.8) | 212.7 | 214.9 | (11.6) | (31.7) | 171.6 |
| Intangible asset amortization and contingent consideration ⁽¹⁾ | 22.3 | (15.1) | (7.2) | — | (44.0) | (15.1) | 59.1 | — |
| Impairment of intangible assets ⁽²⁾ | — | — | — | — | 599.1 | — | (599.1) | — |
| Interest expense, net | (14.1) | 14.1 | — | — | (16.9) | 16.9 | — | — |
| Benefit from income taxes | (16.7) | 16.7 | — | — | (170.0) | 170.0 | — | — |
| GAAP Net Loss/non-GAAP Income | | | | | | | | |
| (Loss) | (53.1) | 36.2 | 77.9 | 61.0 | (502.1) | (114.0) | 604.4 | (11.7) |

1. Amounts for the three and six months ended June 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 for the three and six months ended June 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. Represents the one-time upfront payment related to the License and Settlement Agreement entered into with Sarepta Therapeutics, Inc. in July 2017, subsequent to the second quarter.

Contact:

Investors:

Media:

Traci McCarty

Debra Charlesworth

BioMarin Pharmaceutical Inc.

BioMarin Pharmaceutical Inc.

(415) 455-7558

(415) 455-7451

SOURCE BioMarin Pharmaceutical Inc.

<https://investors.biomin.com/2017-08-02-BioMarin-Announces-Second-Quarter-2017-Financial-Results>