

# BioMarin Announces Second Quarter 2018 Results

- Total Revenues of \$372.8 million in the Quarter
- Received Approval of its Seventh Commercial Product, Palynziq™ Injection for Treatment of Adults with Phenylketonuria
- Provided Two Years of Clinical Data from Ongoing Phase 1/2 Study in Valoctocogene Roxaparvec Gene Therapy for Severe Hemophilia; Amended Phase 3 Protocol to Evaluate Superiority to the Current Standard of Care

SAN RAFAEL, Calif., Aug. 2, 2018 /PRNewswire/ --

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

	Three Months Ended June 30,			Six Months Ended June 30,		
	2018	2017	% Change	2018	2017	% Change
Total Revenues	\$ 372.8	\$ 317.4	17 %	\$ 746.3	\$ 621.2	20 %
Vimizim Net Product Revenues	127.6	103.2	24 %	244.7	209.0	17 %
Kuvan Net Product Revenues	109.0	102.0	7 %	208.1	194.3	7 %
Naglazyme Net Product Revenues	91.1	85.7	6 %	166.1	166.3	—
Aldurazyme Net Product Revenues	24.0	19.9	21 %	90.1	39.3	129 %
Brineura Net Product Revenues	10.9	0.3	n/a	17.8	0.3	n/a
GAAP Net Loss	\$ (16.8)	\$ (36.8)		\$ (60.9)	\$ (53.1)	
GAAP Net Loss per Share - Basic and Diluted	\$ (0.09)	\$ (0.21)		\$ (0.35)	\$ (0.31)	
Non-GAAP Income <sup>(1)</sup>	\$ 19.9	\$ 26.6		\$ 41.2	\$ 61.0	
	<b>June 30, 2018</b>	<b>December 31, 2017</b>				
Cash, cash equivalents and investments	\$ 1,643.1	\$ 1,781.7				

- (1) Non-GAAP Income 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. 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 information reported under Generally Accepted Accounting Principles in the United States (US GAAP).

BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) (BioMarin or the Company) today announced financial results for the second quarter ended June 30, 2018. The financial results that follow represent a comparison of the second quarter of 2018 to the second quarter of 2017. Total revenues were \$372.8 million for the quarter ended June 30, 2018 compared to \$317.4 million for the quarter ended June 30, 2017, an increase of 17%. GAAP Net Loss for the quarter ended June 30, 2018 was \$16.8 million, or \$0.09 loss per basic and diluted share compared to GAAP Net Loss of \$36.8 million, or \$0.21 loss per basic and diluted share for the quarter ended June 30, 2017.



Net product sales for the second quarter of 2018 were \$367.8 million, compared to \$315.9 million in the second quarter of 2017. The increase in net product sales was attributed to the following:

- Vimizim: increased by \$24.4 million, or 24%, due primarily to an increase in the number of commercial patients;
- Brineura: launched in the second quarter of 2017, and contributed \$10.6 million to increased net product revenues;
- Kuvan: increased \$7.0 million, or 7%, driven by an increase in the number of commercial patients;
- Naglazyme: increased \$5.4 million, or 6%, primarily due to government ordering patterns in certain countries and an increase in the number of commercial patients; and
- Aldurazyme: increased \$4.1 million, or 21%, primarily as a result of increased volume.

The decrease in GAAP Net Loss was primarily due to the following:

- Increased gross profit primarily driven by increased Vimizim, Brineura and Kuvan net product revenues; and
- A gain on the sale of intangible assets totaling \$20.0 million received from Pfizer Inc. (Pfizer) associated with the achievement of two regulatory milestones, triggered by the U.S. Food and Drug Administration (FDA) acceptance of the New Drug Application submission for talazoparib (which BioMarin sold to Medivation Inc. prior to Pfizer's acquisition of Medivation Inc.) and the European Medicines Agency acceptance of Pfizer's submission of a Marketing Authorization Application for talazoparib; partially offset by
- Higher research and development (R&D) expense for the expansion of our clinical programs related to traletsinidase alfa (formally referred to as BMN-250), vosoritide and valoctocogene roxaparvec and higher selling, general and administrative (SG&A) expense in support of the preparation for the commercial launch of Palynziq (formerly known as pegvaliase) and the continued commercial expansion of Brineura.

Non-GAAP Income for the second quarter of 2018 was \$19.9 million, compared to Non-GAAP Income of \$26.6 million in the second quarter of 2017. The decrease in Non-GAAP income in the second quarter of 2018 is primarily attributed to higher R&D and SG&A expenses, partially offset by increased gross profit from revenues.

As of June 30, 2018, BioMarin had cash, cash equivalents and investments totaling approximately \$1.6 billion, as compared to \$1.8 billion on December 31, 2017. Our 0.75% senior subordinated convertible notes due 2018 are scheduled to mature in October 2018. Holders have the right to convert their notes at maturity, and with respect to any such conversions we have elected to settle in cash up to the principal amount of the notes, shares of our common stock in respect of conversion value in excess thereof, and cash in lieu of any fractional shares.

Commenting on second quarter results, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "In the quarter, BioMarin achieved numerous value-creating events on both the commercial and regulatory fronts. On May 24 we received standard FDA approval of Palynziq, an important new therapy that helps address a significant unmet need in adults with phenylketonuria (PKU) who have been unable to control their blood Phe levels with current treatment options. We are very happy with the pace of the initial commercial launch of Palynziq and look forward to providing an update on launch metrics in the third quarter of this year."

"In clinical development, we provided two years of clinical data with the 6e13 vg/kg dose of valoctocogene roxaparvec gene therapy for severe Hemophilia A from the ongoing Phase 1/2 study in at the World Federation of Hemophilia (WFH) 2018 World Congress in Glasgow, Scotland. The updated data demonstrated the elimination of

need for prophylaxis and no spontaneous bleeds in two years. In addition, we amended the protocol for the global GENE8-1 (Phase 3) pivotal study to evaluate superiority compared to the current standard of care. The number of participants has been increased to 130 and we now anticipate completing enrollment during the first quarter of 2019."

Mr. Bienaimé continued, "With seven commercial products expected to generate approximately \$1.5 billion in revenues this year, two potentially \$1.0 billion plus late-stage clinical product opportunities, whose pivotal studies are expected to be fully enrolled later this year and in Q1 2019, I believe BioMarin's commercial prospects have never been better."

#### **Updated August 2, 2018 Full-Year Financial Guidance (\$ in millions, except %)**

<b>Item</b>	<b>2018 Guidance</b>	<b>August 2, 2018</b>
Total Revenues	\$1,470 to \$1,530	Unchanged
Kuvan Net Product Revenues	\$440 to \$480	Unchanged
Naglazyme Net Product Revenues	\$325 to \$355	Unchanged
Vimizim Net Product Revenues	\$460 to \$500	Unchanged
Brineura Net Product Revenues	\$35 to \$55	Unchanged
Cost of Sales (% of Total Revenues)	20.0% to 21.0%	Unchanged
Research and Development Expense	\$645 to \$685	\$680 to \$710
Selling, General and Admin. Expense	\$575 to \$615	Unchanged
GAAP Net Loss	\$(115) to \$(165)	Unchanged
Non-GAAP Income *	\$100 to \$140	Unchanged

\*All Financial Guidance items are calculated based on US GAAP with the exception of Non-GAAP Income. 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.

#### **Key Program Highlights**

- **Palynziq for phenylketonuria:** On May 24, 2018, the Company announced that it had received standard approval from the FDA for Palynziq, an injection to reduce blood Phe concentrations in adult patients with phenylketonuria, who have uncontrolled blood Phe concentrations greater than 600 micromoles/L on existing management. Palynziq, a PEGylated recombinant phenylalanine ammonia lyase enzyme, is the first approved enzyme substitution therapy to target the underlying cause of PKU by helping the body to break down Phe. In the U.S., Palynziq is only available through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called the Palynziq REMS Program. Palynziq is BioMarin's second approved treatment for this important condition and its seventh commercialized product.

In March 2018, the European Medicines Agency accepted BioMarin's submission of a Marketing Authorization Application for Palynziq.

- **Valoctocogene roxaparvovec gene therapy for hemophilia A:**

On May 22, the Company provided an update to its previously reported results of an open-label Phase 1/2 study of valoctocogene roxaparvovec, an investigational gene therapy treatment for severe hemophilia A. The updated results were presented during an oral presentation at the WFH 2018 World Congress in Glasgow, Scotland by Dr. John Pasi, M.B., Ch.B., Ph.D., from Barts and The London School of Medicine and Dentistry and primary investigator for this Phase 1/2 study. The data presented at WFH is the most current data (April 16, 2018 cut off) and includes 104 weeks of data for the 6e13 vg/kg cohort and 52 weeks of data for the 4e13 vg/kg cohort.

In the 6e13 vg/kg cohort, the data showed continued and substantial reductions in bleeding requiring Factor VIII infusions with a 97% reduction in mean Annualized Bleed Rate (ABR), with no spontaneous bleeds and elimination of all bleeds in target joints in the second year. 71% and 86% of participants had zero bleeds requiring Factor VIII infusions in years 1 and 2 respectively compared to 14%, who had zero bleeds requiring Factor VIII infusions for a year at baseline. There was a 96% reduction in mean FVIII usage through week 104. Mean Factor VIII activity levels from week 20 through 104 were consistently within the normal or near normal range and no participant was above the upper limit of normal at week 104, expressed as a percentage of normal factor activity in blood. At 104 weeks post-infusion, mean Factor VIII activity level of the 6e13 vg/kg cohort was within the normal range at 59%, and the median was near normal at 46%.

Quality of life as measured by the six-domain Haemo-QoL-A instrument rapidly improved across all domains by up to 17.3 points in mean over baseline through the second year. This is well above the 5.2 point increase considered to be the minimal clinically important difference. The next data update on the Phase 1/2 study with the 6e13 vg/kg cohort is expected to be at the end of year three, in the May 2019 timeframe.

The 4e13 vg/kg cohort also showed a substantial reduction in bleeding requiring Factor VIII infusions with a 92% reduction in ABR. 83% of participants had zero bleeds requiring Factor VIII infusions following treatment for a year compared to 17%, who had zero bleeds requiring Factor VIII infusions for a year at baseline. Mean Factor VIII usage decreased by 98%. Consistent with the reduction in ABR and FVIII usage, quality of life showed mean improvement by 3.8 to 6.3 points.

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). Enrollment completion in the newly amended GENE8-1 study is now expected in the first quarter of 2019. GENE8-2, the ongoing Phase 3 study with the 4e13 vg/kg dose, remains unchanged with an N=40 and is expected to complete enrollment one to two quarters after GENE8-1 in 2019.

The Company updated the protocol for the GENE8-1 study evaluating the 6e13 vg/kg dose and has powered the study to evaluate superiority to the current standard of care, Factor VIII prophylaxis. The study will now include 130 participants (an increase of 90 from the original 40).

During the quarter, the Company began a Phase 1/2 study with the 6e13vg/kg dose of valoctocogene roxaparvovec in up to approximately 10 participants with pre-existing AAV5 antibodies. In addition to that study and the ongoing global Phase 3 studies, GENE8-1 and GENE8-2, BioMarin has two additional clinical studies underway in its comprehensive gene therapy program for the treatment of severe hemophilia A. One to study seroprevalence in people with severe hemophilia A and one non-interventional study to determine baseline characteristics in people with hemophilia A, are ongoing around the world. Also, participants in the Phase 1/2 dose escalation study updated in the quarter at WFH will continue to be monitored as part of the global program underway.

- **Vosoritide for achondroplasia:** On May 11, 2018, the FDA held an advisory committee meeting to discuss drug development for the treatment of children with achondroplasia, the most common form of disproportionate short stature in humans. It was a joint meeting of the Pediatric Advisory Committee and Endocrinologic and Metabolic Drugs Advisory Committee. The committees agreed that annualized growth velocity is the appropriate primary endpoint to use in clinical trials for the treatment of achondroplasia. The committees also expressed support for evaluating secondary endpoints in drug development for achondroplasia including, quality of life, and effects on sleep apnea, hearing loss, and ear infection.

BioMarin's Phase 3 product candidate, vosoritide, an analog of C-type natriuretic peptide (CNP), is being studied in children with achondroplasia. 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 a mean cumulative height 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 baseline study is fully enrolled and the Company expects to complete enrollment of the Phase 3 study in September or October of this year. In addition, the Company expects to have over 5 years of clinical data from the long-term, open-label Phase 2 program to corroborate maintenance of effect.

Given the importance of early intervention in this indication, on June 14, 2018, the Company began an infant/toddler study in 0 to 5 year-old children and dosed the first participant in the global Phase 2 study. Finally, the Company has undertaken a Natural History program to augment clinical understanding of outcomes of untreated patients for comparison to treated patients.

#### **Other Ongoing Clinical Development Programs:**

- **Tralesinidase alfa (formerly referred to as BMN 250) for MPS IIIB (Sanfilippo Syndrome, Type B):** Earlier in the year, at the *WORLD Symposium 2018*, the Company updated preliminary results from the Phase 1/2 trial with tralesinidase alfa, 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). These data suggest that tralesinidase alfa, which is administered via intracerebroventricular (ICV) infusion, reaches the peripheral circulation and has activity in somatic organs. Development Quotient (DQ), a measure of cognitive function normalized to age, was also monitored. Preliminary data suggest stabilization of cognitive DQ at the high dose of tralesinidase alfa in some subjects. Patients with untreated Sanfilippo B syndrome usually show progressive decline in DQ.
- **BMN 290 for Friedreich's Ataxia:** In late 2017, BioMarin announced that it had selected as its next clinical 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 associated with a deficiency in frataxin. Prior to the lead compound being acquired by BioMarin from Repligen Corporation (Repligen), it demonstrated increases in frataxin in Friedreich's ataxia patients. In preclinical models conducted by BioMarin, BMN 290, a compound derived from the original Repligen compound, increases frataxin message expression in brain and cardiac tissues more than two-fold. Currently, there are no approved disease modifying therapies for Friedreich's ataxia. The Company expects to complete IND-enabling evaluations with a view to submitting an IND application for BMN 290 during the second half of 2018.
- **Gene therapy product candidate for phenylketonuria (PKU):** In April 2018, the Company announced that it expects to submit an investigational new drug (IND) application for a gene therapy product for the treatment of PKU in 2019 (after BMN 290 for Friedreich's ataxia). PKU is an autosomal recessive disorder in which phenylalanine hydroxylase, the enzyme that metabolizes the amino acid phenylalanine (Phe), is deficient. PKU leads to high levels of neurotoxic phenylalanine, which affects neurocognitive development and function, if left untreated. In preclinical models, BioMarin's PKU gene therapy product candidate demonstrated sustained, normalized Phe levels in an ongoing study and out to 60 weeks at the last observation. The product candidate will be an AAV vector containing the DNA sequence that codes for the phenylalanine hydroxylase enzyme that is deficient in people with PKU.

BioMarin will host a conference call and webcast to discuss second quarter 2018 financial results today, Thursday, August 2, 2018 at 4:30 p.m. ET. This event can be accessed on the investor section of the BioMarin website at [www.biomarin.com](http://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
Conference ID: 3364409	Conference ID: 3364409

#### **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 seven approved products and multiple clinical and pre-clinical product candidates. For additional information, please visit [www.biomarin.com](http://www.biomarin.com).

#### **Forward-Looking Statements**

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; BioMarin's commercial prospects, including its expected \$1.0 billion plus late-stage clinical product opportunities; the commercial launch of Palynziq; 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 roxaparovec; the ongoing Phase 2 and Phase 3 studies of vosoritide and the Phase 1/2 study of tralesinidase alfa (formerly referred to as BMN 250); the continued clinical development and commercialization of BioMarin's commercial products and product candidates; including BioMarin's plans to file an IND for BMN 290 in the second half of 2018 and an IND for its new gene therapy candidate for the treatment of PKU in 2019; the possible approval and commercialization of BioMarin's product candidates. 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; the introduction of generic versions of BioMarin's commercial products, in particular generic versions of Kuvan; 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, 2018 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<sup>®</sup>, Brineura<sup>®</sup>, Firdapse<sup>®</sup>, Kuvan<sup>®</sup>, Naglazyme<sup>®</sup> and Vimizim<sup>®</sup> are registered trademarks of BioMarin Pharmaceutical Inc., or its affiliates. Palynziq<sup>™</sup> is our trademark. Aldurazyme<sup>®</sup> is a registered trademark of BioMarin/Genzyme LLC.

Contact:

Investors:

Media:

**BIOMARIN PHARMACEUTICAL INC.**

**CONDENSED CONSOLIDATED BALANCE SHEETS**

**June 30, 2018 and December 31, 2017**

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

	<b>June 30, 2018 <sup>(1)</sup></b>	<b>December 31, 2017 <sup>(2)</sup></b>
<b>ASSETS</b>	<b>(unaudited)</b>	
Current assets:		
Cash and cash equivalents	\$ 427,411	\$ 598,028
Short-term investments	935,662	797,940
Accounts receivable, net	363,566	261,365
Inventory	473,356	475,775
Other current assets	80,072	74,036
Total current assets	<u>2,280,067</u>	<u>2,207,144</u>
Noncurrent assets:		
Long-term investments	279,988	385,785
Property, plant and equipment, net	900,480	896,700
Intangible assets, net	502,295	517,510
Goodwill	197,039	197,039
Deferred tax assets	425,380	399,095
Other assets	39,430	29,852
Total assets	<u>\$ 4,624,679</u>	<u>\$ 4,633,125</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 358,732	\$ 401,921
Short-term convertible debt, net	369,752	360,949
Short-term contingent acquisition consideration	76,466	53,648
Total current liabilities	<u>804,950</u>	<u>816,518</u>
Noncurrent liabilities:		
Long-term convertible debt, net	821,871	813,521
Long-term contingent acquisition consideration	57,674	135,318
Other long-term liabilities	55,080	59,105
Total liabilities	<u>1,739,575</u>	<u>1,824,462</u>
Stockholders' equity:		
Common stock, \$0.001 par value: 500,000,000 shares authorized; 177,508,163 and 175,843,749 shares issued and outstanding, respectively.	178	176
Additional paid-in capital	4,577,300	4,483,220
Company common stock held by Nonqualified Deferred Compensation Plan	(13,390)	(14,224)
Accumulated other comprehensive loss	(1,129)	(22,961)
Accumulated deficit	(1,677,855)	(1,637,548)
Total stockholders' equity	<u>2,885,104</u>	<u>2,808,663</u>
Total liabilities and stockholders' equity	<u>\$ 4,624,679</u>	<u>\$ 4,633,125</u>

- (1) As of January 1, 2018, the Company adopted the requirements of Accounting Standards Codification 606, *Revenue from Contracts with Customers* (ASC 606), using the modified retrospective method, and as a result, there is a lack of comparability of certain amounts to the prior periods presented.
- (2) December 31, 2017 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, 2017, filed with the U.S. Securities and Exchange Commission on February 26, 2018.

**BIOMARIN PHARMACEUTICAL INC.**

**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**

**Three and Six Months Ended June 30, 2018 and 2017**

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

**(Unaudited)**

	<b>Three Months Ended June 30,</b>		<b>Six Months Ended June 30,</b>	
	<b>2018 <sup>(1)</sup></b>	<b>2017</b>	<b>2018 <sup>(1)</sup></b>	<b>2017</b>
<b>REVENUES:</b>				
Net product revenues	\$ 367,786	\$ 315,926	\$ 736,885	\$ 618,116
Royalty and other revenues	5,059	1,522	9,407	3,077
Total revenues	<u>372,845</u>	<u>317,448</u>	<u>746,292</u>	<u>621,193</u>
<b>OPERATING EXPENSES:</b>				
Cost of sales				

Research and development	179,082	146,009	169,330	206,042
Selling, general and administrative	153,280	143,505	291,616	263,524
Intangible asset amortization and contingent consideration	10,227	13,411	23,429	22,336
Gain on sale of intangible assets	(20,000)	—	(20,000)	—
Total operating expenses	398,108	356,260	815,927	680,213
<b>LOSS FROM OPERATIONS</b>	(25,263)	(38,812)	(69,635)	(59,020)
Equity in the loss of BioMarin/Genzyme LLC	(107)	(220)	(39)	(743)
Interest income	5,569	2,983	10,803	6,055
Interest expense	(12,225)	(10,040)	(23,787)	(20,159)
Other income, net	2,849	543	2,677	4,015
<b>LOSS BEFORE INCOME TAXES</b>	(29,177)	(45,546)	(79,981)	(69,852)
Benefit from income taxes	(12,385)	(8,713)	(19,040)	(16,729)
<b>NET LOSS</b>	\$ (16,792)	\$ (36,833)	\$ (60,941)	\$ (53,123)
<b>NET LOSS PER SHARE, BASIC AND DILUTED</b>	\$ (0.09)	\$ (0.21)	\$ (0.35)	\$ (0.31)
Weighted average common shares outstanding, basic and dilutive	176,873	174,374	176,405	173,547

(1) As of January 1, 2018, the Company adopted the requirements of ASC 606 using the modified retrospective method, and as a result, there is a lack of comparability of certain amounts to the prior periods presented.

#### Non-GAAP Information

The results presented in this press release for the three and six months ended June 30, 2018 and 2017 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, in certain periods, certain other specified items, as detailed below when applicable. In addition, BioMarin includes in this press release the effects of these adjustments on certain components of GAAP Net Loss for each of the periods presented. In this regard, Non-GAAP Income (Loss) and its components, including Non-GAAP Cost of Sales, Non-GAAP Research and Development expenses, Non-GAAP Selling, General and Administrative expense, Non-GAAP Intangible Asset Amortization and Contingent Consideration, Non-GAAP Gain on the Sale of Intangible Asset and Non-GAAP 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. BioMarin also uses Non-GAAP Income (Loss) internally to understand, manage and evaluate its business and to make operating decisions, and compensation of executives is based in part on this measure.

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:

#### Reconciliation of GAAP Net Loss to Non-GAAP Income (In millions of U.S. dollars) (unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,		Year Ending December 31, 2018
	2018	2017	2018	2017	Guidance
<b>GAAP Net Loss</b>	\$ (16.8)	\$ (36.8)	\$ (60.9)	\$ (53.1)	\$ (115.0) - \$(165.0)
Interest expense, net	6.7	7.0	13.0	14.1	25.0 - 35.0
Benefit from income taxes	(12.3)	(8.7)	(19.0)	(16.7)	(40.0) - 0.0
Depreciation expense	13.5	11.7	29.5	23.7	50.0 - 60.0
Amortization expense	7.5	7.6	15.1	15.1	30.0
Stock-based compensation expense	38.6	40.0	75.2	70.7	150.0 - 170.0
Contingent consideration expense	2.7	5.8	8.3	7.2	20.0 - 30.0
Gain on sale of intangible assets	(20.0)	—	(20.0)	—	(20.0)
Non-GAAP Income	\$ 19.9	\$ 26.6	\$ 41.2	\$ 61.0	\$100 - \$140

The following reconciliation of the GAAP reported to the 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 June 30.	
2018	2017
Adjustments	Adjustments

	<b>GAAP Reported</b>	Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non- GAAP	<b>GAAP Reported</b>	Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non- GAAP
Cost of sales	\$ 79.1	\$ —	\$ (3.3)	\$ 75.8	\$ 56.3	\$ —	\$ (2.5)	\$ 53.8
Research and development	175.6	(7.9)	(15.5)	152.2	143.0	(6.2)	(14.6)	122.2
Selling, general and administrative	153.3	(5.6)	(19.8)	127.9	143.5	(5.5)	(22.9)	115.1
Intangible asset amortization and contingent consideration	10.2	(7.5)	(2.7)	—	13.4	(7.6)	(5.8)	—
Gain on sale of intangible assets	(20.0)	—	20.0	—	—	—	—	—
Interest expense, net	(6.7)	6.7	—	—	(7.0)	7.0	—	—
Benefit from income taxes	(12.3)	12.3	—	—	(8.7)	8.7	—	—
GAAP Net Loss/Non-GAAP Income	(16.8)	15.4	21.3	19.9	(36.8)	17.6	45.8	26.6

**Six Months Ended June 30,**

	<b>2018</b>				<b>2017</b>			
	<b>GAAP Reported</b>	<b>Adjustments</b>		Non- GAAP	<b>GAAP Reported</b>	<b>Adjustments</b>		Non- GAAP
		Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments			Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	
Cost of sales	\$ 161.4	\$ —	\$ (6.4)	\$ 155.0	\$ 106.3	\$ —	\$ (4.8)	\$ 101.5
Research and development	359.5	(18.4)	(28.8)	312.3	288.0	(12.7)	(26.1)	249.2
Selling, general and administrative	291.6	(11.1)	(40.0)	240.5	263.5	(11.0)	(39.8)	212.7
Intangible asset amortization and contingent consideration	23.4	(15.1)	(8.3)	—	22.3	(15.1)	(7.2)	—
Gain on sale of intangible assets	(20.0)	—	20.0	—	—	—	—	—
Interest expense, net	(13.0)	13.0	—	—	(14.1)	14.1	—	—
Benefit from income taxes	(19.0)	19.0	—	—	(16.7)	16.7	—	—
GAAP Net Loss/Non-GAAP Income (Loss)	(60.9)	38.6	63.5	41.2	(53.1)	36.2	77.9	61.0

SOURCE BioMarin Pharmaceutical Inc.

<http://investors.biomin.com/2018-08-02-BioMarin-Announces-Second-Quarter-2018-Results>