

**BioMarin Announces First Quarter 2018 Financial Results**

**- Total Revenues Top \$373 million in the Quarter; GAAP Net Loss of \$44 million; Non-GAAP Income of \$21 million**

**- Subjects in Gene Therapy Pivotal Studies for Hemophilia A to Receive Valoctocogene Roxaparvec Manufactured at New BioMarin Commercial Facility**

**- Gene Therapy Product for the Treatment of Phenylketonuria Selected as New IND Candidate for 2019**

SAN RAFAEL, Calif., April 25, 2018 [/PRNewswire/](#) --

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

	<b>Three Months Ended March 31,</b>		
	<b>2018</b>	<b>2017</b>	<b>% Change</b>
Total Revenues	\$ 373.4	\$ 303.7	23%
Vimizim Net Product Revenues	117.1	105.8	11%
Kuvan Net Product Revenues	99.1	92.3	7%
Naglazyme Net Product Revenues	75.0	80.6	(7)%
Aldurazyme Net Product Revenues	66.1	19.4	241%
Brineura Net Product Revenues	6.9	—	n/a
GAAP Net Loss	\$ (44.1)	\$ (16.3)	
GAAP Net Loss per Share - Basic	\$ (0.25)	\$ (0.09)	
GAAP Net Loss per Share - Diluted	\$ (0.26)	\$ (0.09)	
Non-GAAP Income <sup>(1)</sup>	\$ 21.3	\$ 34.3	
	<b>March 31,</b>	<b>December 31,</b>	
	<b>2018</b>	<b>2017</b>	
Cash, cash equivalents and investments	\$ 1,696.4	\$ 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 GAAP reported information.

BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) today announced financial results for the first quarter ended March 31, 2018. For the quarter ended March 31, 2018 GAAP Net Loss was \$44.1 million, or \$0.25 loss per basic and \$0.26 loss per diluted share, respectively, compared to GAAP Net Loss of \$16.3 million, or \$0.09 loss per basic and diluted share, respectively, for the quarter ended March 31, 2017. The increase in GAAP Net Loss year over year was primarily due to higher research and development expenses for expansion of our clinical programs related to BMN-250, valoctocogene roxaparvec and vosoritide, largely offset by higher gross profit from increased Aldurazyme and Kuvan net product revenues. In addition, higher selling, general and administrative expenses in support of the ongoing commercial launch of Brineura and for the anticipated launch of pegvaliase contributed to the increase in GAAP Net Loss in the quarter compared to the same period last year.

Non-GAAP Income for the quarter ended March 31, 2018 was \$21.3 million, compared to Non-GAAP Income of \$34.3 million for the quarter ended March 31, 2017. Similar to the GAAP results for the quarter ended March 31, 2018, the decrease in Non-GAAP Income was primarily due to increased research and development expenses, partially offset by increased gross margins on net product revenues, as well as higher selling, general and administrative expenses compared to the same period last year.

Total revenues were \$373.4 million for the quarter ended March 31, 2018 compared to \$303.7 million for the quarter ended March 31, 2017, an increase of 23%. For the quarter ended March 31, 2018, Aldurazyme net product revenues increased \$46.7 million year over year, or 241%, of which \$27.2 million is due to the different revenue recognition principles applied as a result of our adoption of Accounting Standards Codification 606 *Revenue from Contracts with Customers*, (ASC 606), and \$19.5 million due to the timing of product sales to Genzyme. Vimizim net product revenues increased by \$11.3 million, or 11%, year over year, due primarily to an increase of 16% in the number of Vimizim commercial patients. Kuvan net product revenues increased \$6.8 million, or 7%, year over year, driven by a 9% increase in the number of commercial patients on Kuvan therapy in North America and continued growth in ex-North American territories. Naglazyme net product revenues decreased \$5.6 million, or 7%, year over year during the quarter ended March 31, 2018, primarily due to the timing of government orders in certain countries. The number of Naglazyme commercial patients increased 4% year over year.

As of March 31, 2018, BioMarin had cash, cash equivalents and investments totaling approximately \$1.7 billion, as compared to \$1.8 billion on December 31, 2017.

Commenting on first quarter results, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "2018 is a year of execution as we aim to achieve numerous value-creating catalysts across the business. In clinical development, we intend to complete enrollment of our global GENE8-1 pivotal study with the only late-stage gene therapy product for the treatment of Hemophilia A, valoctocogene roxaparvovec. Concurrently, we continue to anticipate reaching roughly \$1.5 billion in Total Revenues for the full-year 2018, per our guidance. On the regulatory front, following the acceptance of our Biologics License Application for pegvaliase for the treatment of phenylketonuria (PKU), which received a Priority Review designation, we anticipate FDA action by the end of May 2018. In the quarter, we were also pleased to have submitted the Marketing Authorization Application for pegvaliase in Europe."

Mr. Bienaimé continued, "The adult PKU market is primed for an efficacious treatment option that lowers Phe while allowing for near normal protein intake. We understand that the PKU community is anxiously awaiting the potential approval of pegvaliase given the dramatic phenylalanine (Phe) reductions observed in our Phase 3 studies. We are very excited to be on the cusp of the second potential product approval in less than two years, following the approval of Brineura in 2017. On the heels of a potential pegvaliase approval, we are also thrilled to announce our next gene therapy program for PKU that leverages both our expertise developing and manufacturing AAV products as well as our long-established commercial footprint selling Kuvan. We are not aware of any other company of our size that has six commercial products, a stable of potential blockbuster late-stage clinical product candidates and over \$1 billion in annual revenues. I believe BioMarin is unique in our industry and that we are only beginning to unlock the value of both our base business and development pipeline."

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

#### **Total Revenues**

	<b>Three Months Ended March 31,</b>			
	<b>2018</b>	<b>2017</b>	<b>\$ Change</b>	<b>% Change</b>
Vimizim	\$ 117.1	\$ 105.8	\$ 11.3	11%
Kuvan	99.1	92.3	6.8	7%
Naglazyme	75.0	80.6	(5.6)	(7)%
Aldurazyme <sup>(1)</sup>	66.1	19.4	46.7	241%
Brineura	6.9	—	6.9	n/a
Firdapse	4.9	4.1	0.8	20%
Net Product Revenues	369.1	302.2	66.9	22%
Royalty and Other Revenues	4.3	1.5	2.8	
Total Revenues	\$ 373.4	\$ 303.7	\$ 69.7	23%

(1) The ASC 606 *Revenue from Contracts with Customers* accounting standard was adopted using the modified retrospective approach. As such, prior period revenues have not been restated and therefore Aldurazyme revenues are not comparable for the periods presented.

#### **Effect on Aldurazyme Net Product Revenues for the Adoption of ASC 606**

Effective January 1, 2018, the Company adopted ASC 606, which provides principles for recognizing revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the Company expects to be entitled in exchange for those goods or services. The Company adopted ASC 606 on a modified retrospective basis through a cumulative adjustment of a \$20.0 million reduction to Accumulated Deficit, a \$26.0 million increase in Accounts Receivable and a \$6.0 million decrease in Deferred Tax Assets.

The adoption of ASC 606 did not have an impact on the timing of revenue recognition for the products directly marketed by BioMarin. However, under the new guidance, we now recognize Aldurazyme revenues under our agreement with Genzyme at the point in time when control of the product is transferred from BioMarin to Genzyme. The amount of revenue recognized is based on the entire estimated payment that BioMarin expects to receive when the product is sold by Genzyme to its customers. Under the former revenue accounting standards, we only recognized \$100.00 per vial when product was transferred to Genzyme and did not recognize the variable consideration component until Genzyme had sold the product. This change in accounting standard contributed \$27.2 million to the increase in Aldurazyme revenues in the first quarter. Any variances between the amounts that we estimate to receive at the time of our product transfers to Genzyme and the actual payments that we ultimately receive from Genzyme based on their sales of the product will affect Net Product Revenues and earnings in the period such variances become known, although such amounts are not expected to be material.

#### **Reaffirmed 2018 Full-Year Financial Guidance (\$ in millions, except %), as presented February 22, 2018**

<b>Item</b>	<b>2018 Guidance</b>
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
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 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**

- **Gene therapy product candidate for phenylketonuria (PKU):**

Today, the Company announced that a gene therapy product will be the next IND (after BMN 290 for Friedreich's ataxia) candidate for the treatment of PKU in 2019. 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 would affect neurocognitive development, if left untreated. In preclinical models, BioMarin's PKU gene therapy product candidate demonstrated sustained, normalized Phe levels without hypophenylalanemia in an ongoing study and out to 53 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.

- **Valoctocogene roxaparvovec (formerly referred to as BMN 270) gene therapy for hemophilia A:**

In December 2017, 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 first patient in the GENE8 pivotal studies to receive valoctocogene roxaparvovec that was manufactured in BioMarin's new commercial gene therapy facility will be dosed in the near future.

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 are open-label single-arm studies to evaluate the efficacy and safety of valoctocogene roxaparvovec. The primary endpoint in both studies will be based on the factor VIII activity level achieved following valoctocogene roxaparvovec, and the secondary endpoints will be annualized factor VIII replacement therapy use rate and annualized bleed rate.

In the second quarter BioMarin intends to begin a Phase 1/2 Study with the 6e13 kg/vg dose and with approximately 10 patients who produce neutralizing antibodies against AAV5.

Also announced today, the Company intends to provide an update on the ongoing Phase 2 program with valoctocogene roxaparvovec at the World Federation of Hemophilia 2018 World Congress next month. BioMarin plans to share a two-year update of the 6e13 vg/kg dose cohort, as well as a one-year update of the 4e13 vg/kg dose cohort.

On March 21, 2018, BioMarin announced that the International Society for Pharmaceutical Engineering selected the Company's gene therapy manufacturing facility as the 2018 Facility of the Year Category Winner for Project Execution. The recognition highlighted the company's successful construction of the facility in Novato, California, which took less than a year to transform basic infrastructure into one of the first gene manufacturing facilities of its kind in the world.

- **Pegvaliase for phenylketonuria (PKU):** On March 28, 2018 the European Medicines Agency accepted BioMarin's submission of a Marketing Authorization Application for pegvaliase. The U.S. Food and Drug Administration accepted the Biologics License Application (BLA) for pegvaliase and granted priority review status in August 2017, with the current Prescription Drug User Fee Act Action Goal Date of May 25, 2018. Pegvaliase is a PEGylated recombinant phenylalanine ammonialyase enzyme product to reduce blood Phe levels in adult patients with PKU who have uncontrolled blood Phe levels on existing management.
- **Vosoritide for achondroplasia:** Vosoritide, an analog of C-type natriuretic peptide (CNP), is being studied 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 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 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 from the dose escalation arm of the study 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 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 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. On the basis of these results, the Company selected an improved candidate, 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. In preclinical models conducted by BioMarin, BMN 290, a compound derived from the original Repligen compound, increases frataxin message expression in brain tissues more than two-fold. 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 will host a conference call and webcast to discuss first quarter 2018 financial results today, Wednesday, April 25, 2018 at 4:30 p.m. ET. This event can be accessed on the investor section of the BioMarin website at [www.biomin.com](http://www.biomin.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: 1665515	Conference ID: 1665515

#### 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](http://www.biomin.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; 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 roxaparvovec; 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 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, including the potential of some of these products to reach blockbuster status; the adequacy of production of valoctocogene roxaparvovec in the Company's

commercial gene therapy manufacturing facility and the imminent dosing of the first patient from product manufactured in this facility; and actions by regulatory authorities, including the expected FDA action on the pegvaliase BLA at the end of May 2018. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: BioMarin's success in the commercialization of its commercial products; Genzyme Corporation's success in continuing the commercialization of Aldurazyme; results and timing of current and planned preclinical studies and clinical trials, BioMarin's ability to successfully manufacture its commercial products and product candidates; the content and timing of decisions by the FDA, the European Commission and other regulatory authorities concerning each of the described products and product candidates; the market for each of these products; actual sales of BioMarin's commercial products; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission (SEC), including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's Annual Report on Form 10-K for the year ended December 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<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. Aldurazyme<sup>®</sup> is a registered trademark of BioMarin/Genzyme LLC.

**BIOMARIN PHARMACEUTICAL INC.**

**CONDENSED CONSOLIDATED BALANCE SHEETS**

**March 31, 2018 and December 31, 2017**

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

	<b>March 31, 2018 <sup>(1)</sup></b>	<b>December 31, 2017 <sup>(2)</sup></b>
<b>ASSETS</b>	(unaudited)	
Current assets:		
Cash and cash equivalents	\$ 473,980	\$ 598,028
Short-term investments	908,815	797,940
Accounts receivable, net	318,394	261,365
Inventory	468,161	475,775
Other current assets	71,760	74,036
Total current assets	2,241,110	2,207,144
Noncurrent assets:		
Long-term investments	313,599	385,785
Property, plant and equipment, net	895,392	896,700
Intangible assets, net	509,902	517,510
Goodwill	197,039	197,039
Deferred tax assets	407,009	399,095
Other assets	32,666	29,852
Total assets	\$ 4,596,717	\$ 4,633,125
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 346,538	\$ 401,921
Short-term convertible debt, net	365,326	360,949
Short-term contingent acquisition consideration payable	61,607	53,648
Total current liabilities	773,471	816,518
Noncurrent liabilities:		
Long-term convertible debt, net	817,672	813,521
Long-term contingent acquisition consideration payable	137,618	135,318
Other long-term liabilities	60,953	59,105
Total liabilities	1,789,644	1,924,462

	<del>1,789,714</del>	<del>1,824,462</del>
Stockholders' equity:		
Common stock, \$0.001 par value: 500,000,000 shares authorized; 176,653,480 and 175,843,749 shares issued and outstanding, respectively.	177	176
Additional paid-in capital	4,510,451	4,483,220
Company common stock held by Nonqualified Deferred Compensation Plan	(14,017)	(14,224)
Accumulated other comprehensive loss	(28,545)	(22,961)
Accumulated deficit	(1,661,063)	(1,637,548)
Total stockholders' equity	<u>2,807,003</u>	<u>2,808,663</u>
Total liabilities and stockholders' equity	<u>\$ 4,596,717</u>	<u>\$ 4,633,125</u>

- (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 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 Months Ended March 31, 2018 and 2017**

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

**(Unaudited)**

	<b>Three Months Ended March 31,</b>	
	<b>2018 <sup>(1)</sup></b>	<b>2017</b>
<b>REVENUES:</b>		
Net product revenues	\$ 369,099	\$ 302,190
Royalty and other revenues	4,348	1,555
Total revenues	<u>373,447</u>	<u>303,745</u>
<b>OPERATING EXPENSES:</b>		
Cost of sales	82,333	50,006
Research and development	183,948	145,003
Selling, general and administrative	138,336	120,019
Intangible asset amortization and contingent consideration	13,202	8,925
Total operating expenses	<u>417,819</u>	<u>323,953</u>
<b>LOSS FROM OPERATIONS</b>	<u>(44,372)</u>	<u>(20,208)</u>
Equity in the income (loss) of BioMarin/Genzyme LLC	68	(523)
Interest income	5,234	3,072
Interest expense	(11,562)	(10,119)
Other income (expense)	(172)	3,472
<b>LOSS BEFORE INCOME TAXES</b>	<u>(50,804)</u>	<u>(24,306)</u>
Benefit from income taxes	(6,655)	(8,016)
<b>NET LOSS</b>	<u>\$ (44,149)</u>	<u>\$ (16,290)</u>
<b>NET LOSS PER SHARE, BASIC</b>	<u>\$ (0.25)</u>	<u>\$ (0.09)</u>
<b>NET LOSS PER SHARE, DILUTED</b>	<u>\$ (0.26)</u>	<u>\$ (0.09)</u>
Weighted average common shares outstanding, basic	<u>175,932</u>	<u>172,710</u>

Weighted average common shares outstanding, diluted	176,150	172,710
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(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 to the prior periods presented.

### Non-GAAP Information

The results presented in this press release for the three months ended March 31, 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		Year Ending
	March 31, 2018	2017	December 31, 2018 Guidance
<b>GAAP Net Loss</b>	<b>\$ (44.1)</b>	<b>\$ (16.3)</b>	<b>\$(115.0) - \$(165.0)</b>
Interest expense, net	6.3	7.0	25.0 - 35.0
Benefit from income taxes	(6.7)	(8.0)	(40.0) - 0.0
Depreciation expense	16.0	12.0	50.0 - 60.0
Amortization expense	7.6	7.5	30.0
Stock-based compensation expense	36.6	30.7	150.0 - 170.0
Contingent consideration expense	5.6	1.4	20.0 - 30.0
Gain on sale of intangible asset	—	—	(20.0)
Non-GAAP Income	<u>\$ 21.3</u>	<u>\$ 34.3</u>	<u>\$100 - \$140</u>

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 March 31**

	<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	\$ <b>82.3</b>	\$ —	\$ (3.1)	\$ 79.2	\$ <b>50.0</b>	\$ —	\$ (2.3)	\$ 47.7
Research and development	<b>183.9</b>	(10.5)	(13.3)	160.1	<b>145.0</b>	(6.5)	(11.5)	127.0
Selling, general and administrative	<b>138.3</b>	(5.5)	(20.2)	112.6	<b>120.0</b>	(5.5)	(16.9)	97.6
Intangible asset amortization and contingent consideration	<b>13.2</b>	(7.6)	(5.6)	—	<b>8.9</b>	(7.5)	(1.4)	—
Interest expense, net	<b>(6.3)</b>	6.3	—	—	<b>(7.0)</b>	7.0	—	—
Benefit from income taxes	<b>(6.7)</b>	6.7	—	—	<b>(8.0)</b>	8.0	—	—
GAAP Net Loss/Non-GAAP Income	<b>(44.1)</b>	23.2	42.2	21.3	<b>(16.3)</b>	18.5	32.1	34.5

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