

BioMarin Announces Second Quarter 2019 Financial Results

- Total Revenues of \$387.8 million in the Quarter
- Full-year 2019 Total BioMarin Revenue Guidance of Between \$1.68 billion to \$1.75 billion Reaffirmed
- As of June 30, 2019, 551 U.S. Commercial Patients Receiving Treatment with Palynziq®

SAN RAFAEL, Calif., Aug. 1, 2019 /PRNewswire/ --

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

	Three Months Ended June 30,			Six Months Ended June 30,		
	2019	2018	% Change	2019	2018	% Change
Total Revenues	\$ 387.8	\$ 372.8	4 %	\$ 788.5	\$ 746.3	6 %
Net Product Revenues Marketed by BioMarin (1)	373.3	343.8	9 %	722.5	646.8	12 %
Vimizim Net Product Revenues	122.7	127.6	(4) %	248.5	244.7	2 %
Kuvan Net Product Revenues	113.3	109.0	4 %	220.2	208.1	6 %
Naglazyme Net Product Revenues	98.2	91.1	8 %	185.1	166.1	11 %
Palynziq Net Product Revenues	18.8	—	n/a	31.1	—	n/a
Brineura Net Product Revenues	14.8	10.9	36 %	27.0	17.8	52 %
Aldurazyme Net Product Revenues	5.8	24.0	(76) %	51.1	90.1	(43) %
GAAP Net Loss	\$ (37.4)	\$ (16.8)		\$ (93.9)	\$ (60.9)	
GAAP Net Loss per Share – Basic and Diluted	\$ (0.21)	\$ (0.09)		\$ (0.53)	\$ (0.35)	
Non-GAAP Income (2)	\$ 17.1	\$ 19.9		\$ 42.2	\$ 41.2	

	June 30, 2019	December 31, 2018
Cash, cash equivalents and investments	\$1,106.1	\$1,320.2

- (1) Net Product Revenues Marketed by BioMarin is the sum of revenues from Vimizim, Kuvan, Naglazyme, Palynziq, Brineura and Firdapse, each calculated in accordance with Generally Accepted Accounting Principles in the United States (U.S. GAAP). Sanofi Genzyme (Genzyme) is BioMarin's sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third-parties. Refer to page 8 for a table showing Net Product Revenues by product, including Firdapse.
- (2) Non-GAAP Income is defined by the Company as reported GAAP Net Income, 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 U.S. GAAP.

BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) (BioMarin or the Company) today announced financial results for the second quarter ended June 30, 2019.



Total Net Product Revenues for the second of quarter 2019 increased to \$379.1 million, compared to \$367.8 million for the second quarter of 2018. The increase in Net Product Revenues was attributed to the following:

- Palynziq Net Product Revenues during the second quarter of 2019 totaled \$18.8 million driven primarily by new patients initiating therapy in the U.S. as the product launched in the third quarter of 2018. Palynziq received approval from the U.S. Food and Drug Administration (FDA) in May 2018 and from the European Medicines Agency (EMA) in May 2019. EU commercial sales are expected to commence in the third quarter of 2019; and
- Naglazyme Net Product Revenues increased by \$7.1 million, or 8%, primarily due to increased sales volume driven by government ordering patterns from certain Latin American and European countries, partially offset by
- Aldurazyme Net Product Revenues decreased \$18.2 million, due to the timing of customer acceptance for product shipped to Genzyme in the second quarter for which no revenue was recognized as of June 30, 2019. Aldurazyme revenue recognition is based on timing of Genzyme acceptance of product shipment. Approximately \$23.0 million of Aldurazyme revenue that was shipped in the second quarter is expected to be recognized in the third quarter of 2019 once product has been accepted by Genzyme. The delay was due to a change in the location where Genzyme receives product. Genzyme's Aldurazyme revenues, as provided to BioMarin by Genzyme, increased \$11.6 million or 9% during the six months ended June 30, 2019, compared to the same period in 2018. Full-year total Aldurazyme revenues are expected to be consistent with full-year Aldurazyme revenues in prior years and in the \$100.0 to \$120.0 million range; and
- Vimizim Net Product Revenues decreased by \$4.9 million, or 4%, primarily due to decreased sales volume driven by government ordering patterns in certain Latin American, Middle Eastern and European countries.

The increase in GAAP Net Loss for the second quarter of 2019, compared to the same period in 2018 was primarily due to the following:

- higher research and development (R&D) expense related to preclinical activities for our PKU gene therapy development program and clinical activities for our valoctocogene roxaparvec and vosoritide development programs, offset by decreased R&D expense related to Palynziq for which we began capitalizing manufacturing costs upon FDA approval in May 2018 and a decrease in tralesenidase alfa clinical manufacturing costs. R&D expenses in the quarter were consistent with 2019 guidance despite the acceleration of the valoctocogene roxaparvec development program and subsequent activities implemented to pursue an expedited regulatory path forward; and
- higher intangible asset amortization related to the Palynziq in-process research and development assets that were placed into service following EU approval in May 2019; and
- higher selling, general and administrative (SG&A) expense in support of the EU commercial launch and continued U.S. expansion of Palynziq, pre-commercialization activities related to valoctocogene roxaparvec and increased general and administrative expense primarily attributed to personnel-related costs resulting from increased headcount to support our growth; partially offset by;
- increased gross profits of \$12.9 million driven by increased product sales.

The increase in GAAP Net Loss for the second quarter of 2019 did not affect the Company's full-year GAAP Net Loss Guidance, which remains unchanged.

Non-GAAP Income for the second quarter of 2019 decreased \$2.8 million, or 14%, to \$17.1 million, compared to \$19.9 million for the same period in 2018. The decrease in Non-GAAP Income for the quarter, compared to the same period in 2018, was attributed to higher R&D expense and SG&A expense, partially offset by increased gross profit from sales as described above. The decrease in Non-GAAP Income for the second quarter of 2019 did not affect the Company's full-year Non-GAAP Income Guidance, which remains unchanged.

As of June 30, 2019, BioMarin had cash, cash equivalents and investments totaling approximately \$1.1 billion, as compared to \$1.3 billion on December 31, 2018.

Commenting on second quarter results, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "During the first half of 2019 we laid the foundation for a number of significant milestones anticipated over the coming months. We recently announced our plans to submit marketing applications for valoctocogene roxaparvec gene therapy for severe hemophilia A in both the United States and Europe. We expect to submit both applications in the fourth quarter of this year based on recent interactions with health authorities in those regions. We are pleased to have the opportunity to initiate the first review of a marketing application for any type of hemophilia indication with a gene therapy product. With valoctocogene roxaparvec people with severe hemophilia A may soon have the opportunity to experience improved quality of life, including consequences of bleeding, physical functioning, role functioning, emotional impact, treatment concern, and worry. We are very grateful that health authorities are aligned in their focus to expedite the review of this potentially transformative treatment option given the unmet need with current standard of care."

Mr. Bienaimé continued, "Phase 3 results from another potential commercial product, vosoritide for the treatment of achondroplasia, are expected to read-out at the end of this year. Our newest study, a global Phase 2 with vosoritide in infants and young children (less than 60 months old) with achondroplasia is enrolling very well. We expect all subjects ages 6 months through 5 years to be enrolled by year-end. We have been very pleased with the high-level of enthusiasm from families wanting to participate in this program and look forward to starting enrollment in the youngest cohort, infants up to 6 months old, later this year. Another very significant opportunity that is gaining momentum is the global commercialization of Palynziq. We have been very pleased with the pace of the U.S. launch, as we ended the second quarter with 551 patients on reimbursed Palynziq, and an additional 158 naive patients having completed enrollment and awaiting their first injection. Building on this success and as part of our strategy to increase our leadership in the PKU market, we anticipate the submission of an investigational new drug application (IND) and/or a clinical trial application (CTA) for BMN 307, our gene therapy product for PKU, in the second half of 2019. BMN 307 demonstrated lifetime normalization of Phe in a validated PKU mouse model, and as a result, we believe it has the potential to be an important new treatment and market expander as part of our PKU franchise."

2019 Full-Year Financial Guidance unchanged (in millions, except %)

Item	2019 Guidance			
Total Revenues	\$ 1,680	to	\$ 1,750	
Vimizim Net Product Revenues	\$ 530	to	\$ 570	
Kuvan Net Product Revenues	\$ 420	to	\$ 460	
Naglazyme Net Product Revenues	\$ 350	to	\$ 380	
Palynziq Net Product Revenues	\$ 70	to	\$ 100	
Brineura Net Product Revenues	\$ 55	to	\$ 75	
Cost of Sales (% of Total Revenues)	20	% to	21	%
Research and Development Expense	\$ 740	to	\$ 780	
Selling, General and Administrative Expense	\$ 650	to	\$ 690	
GAAP Net Loss	\$ (45)	to	\$ (85)	
Non-GAAP Income *	\$ 130	to	\$ 170	

* All Financial Guidance items are calculated based on U.S. GAAP with the exception of Non-GAAP Income/Loss. 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 corresponding GAAP reported information.

Key Program Highlights

- **Valoctocogene roxaparvec gene therapy for hemophilia A:** On July 8, the Company announced that based on recent meetings with health authorities in the U.S. and Europe, it plans to submit marketing applications to both the FDA and the European Medicines Agency (EMA) in the fourth quarter of 2019 for its investigational gene therapy, valoctocogene roxaparvec, for adults with severe hemophilia A.

These submissions will be based on the updated three-year Phase 1/2 data and the recently completed Phase 3 interim analysis of patients treated with valoctocogene roxaparvec material from the to-be-commercialized process. Both submissions are expected to represent the first time a gene therapy product for any type of hemophilia indication will be reviewed for marketing authorization by health authorities.

The Company has chosen to cease development of the 4e13 vg/kg dose of valoctocogene roxaparvec given the overwhelming preference by patients to be treated with the 6e13 vg/kg dose. Enrollment continues in the GENE8-1 Phase 3 study and the 52 week results are anticipated at the end of 2020.

- **Palynziq for PKU:** Palynziq, an injection to reduce blood Phe concentrations in adult patients with PKU, was added to BioMarin's commercial product portfolio upon its U.S. approval May 2018. As of June 30, 2019, 551 patients were on reimbursed Palynziq, with an additional 158 naïve patients enrolled and awaiting their first treatment with commercial Palynziq. Of the 551 patients on therapy at the end of the second quarter, 410 were formerly naïve patients and 141 transitioned from clinical studies. Of the 125 PKU clinics in the U.S., 92 unique clinics had at least one complete patient enrollment in the REMS program as of June 30, 2019.

On May 6, 2019, the European Commission (EC) granted marketing authorization for Palynziq at doses of up to 60 milligrams once daily, to reduce blood Phe concentrations in patients with PKU aged 16 and older, who have inadequate blood Phe control (blood Phe levels greater than 600 micromol/L) despite prior management with available treatment options. In addition, the EC acknowledged that the Phase 3 trial and extension study is suggestive of an improvement in inattention and mood symptoms.

- **Vosoritide for children with achondroplasia:** On June 18, 2019, the *New England Journal of Medicine* published the 42 month results from the Phase 2 study with vosoritide in children ages 5 to 14 years. The results also appeared in the July 4 printed issue. BioMarin expects to have over 5 years of clinical data from this Phase 2 study to corroborate maintenance of effect at the time of anticipated marketing application submissions.

The Company expects top line results from the ongoing global, Phase 3 study by year-end 2019. The vosoritide development program includes four distinct areas of focus to support global approval, including a large contemporaneous natural history study which is underway. The global Phase 3 study, which is fully enrolled, is a randomized, placebo-controlled study of vosoritide in approximately 110 children with achondroplasia between the ages of 5 to 14 years.

In 2018, BioMarin began a global Phase 2 study with vosoritide in infants and young children (newborn to 60 months old) with achondroplasia, to determine the impact of treatment in this age group. Three cohorts, segmented by age, are being enrolled in this study. Cohort 1 includes children ages 24 to 60 months old and has completed enrollment. Cohort 2 includes children ages 6 to 24 months old and is expected to complete enrollment by year-end. The Company plans to begin enrolling infants up to 6 months old by year-end.

- **Tralesinidase alfa (formerly referred to as BMN 250) for MPS IIIB (Sanfilippo Syndrome, Type B):** Tralesinidase alfa is currently being evaluated in ongoing natural history and clinical trials. Previously, encouraging signs of biochemical and clinical efficacy have been suggested. Trials are ongoing to collect further data in regard to the untreated natural history of the condition, as well as biochemical and clinical outcomes of therapy.
- **BMN 307 gene therapy product candidate for phenylketonuria (PKU):** As previously announced, the Company expects to submit an IND and/or a CTA for a gene therapy product for the treatment of PKU in the second half of 2019. At R&D Day 2018, BioMarin shared data with BMN 307 that demonstrated a lifetime Phe correction sustained at 80 weeks in preclinical mouse models. BMN 307 is an AAV vector containing the DNA sequence that codes for the phenylalanine hydroxylase enzyme that is deficient in people with PKU. Product to support clinical evaluation will be produced at BioMarin's gene therapy manufacturing facility, where valoctocogene roxaparvovec is currently made, using a commercial scale manufacturing process to facilitate rapid clinical development.
- **BMN 290 for Friedreich's Ataxia:** The Company today announced plans to cease the preclinical studies in the BMN 290 program based on progress of other portfolio assets that have demonstrated stronger product profiles.

BioMarin will host a conference call and webcast to discuss second quarter 2019 financial results today, Thursday, August 1, 2019 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	Replay Dial-in Number: 855.859.2056
International Dial-in Number: 574.990.1362	Replay International Dial-in Number: 404.537.3406
Conference ID: 6989536	Conference ID: 6989536

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare diseases and medical conditions. The Company selects product candidates for diseases and conditions that represent a significant unmet medical need, have well-understood biology and provide an opportunity to be first-to-market or offer a significant benefit over existing products. The Company's portfolio consists of several commercial therapies and multiple clinical and preclinical product candidates.

For additional information, please visit 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, including Aldurazyme revenues expected to be recognized in the third quarter of 2019 once product shipped in the second quarter has been accepted by Sanofi Genzyme (Genzyme) and full year Aldurazyme revenues, Net Product Revenues and expenses for BioMarin's commercial products, Cost of Sales, GAAP Net Loss, Non-GAAP Income and other specified income statement guidance, including guidance for Total Revenues for the full-year; the financial performance of BioMarin as a whole; BioMarin's potential for growth; BioMarin anticipating significant milestones over the coming months; the timing of BioMarin's clinical development and commercial prospects, including (i) BioMarin's planned submissions to regulatory authorities, including marketing authorization applications for valoctocogene roxaparvovec in both the U.S. and Europe and an IND and/or a CTA for BMN 307, (ii) BioMarin's clinical studies and trials, (iii) completion of enrollment of those studies and trials, including enrollment in BioMarin's Phase 3 program with valoctocogene roxaparvovec and global Phase 2 study with vosoritide, and (iv) announcements of data from those studies and trials, including BioMarin's Phase 3 program and Phase 1/2 study with valoctocogene roxaparvovec and global Phase 3 study of vosoritide; the clinical development and commercialization of BioMarin's product candidates and commercial products, including (i) valoctocogene roxaparvovec offering people with severe hemophilia A the opportunity to experience improved quality of life, (ii) BioMarin's planned submission of marketing authorization applications for valoctocogene roxaparvovec representing the first time a gene therapy product for any type of hemophilia will be reviewed for marketing authorization by health authorities, (iii) product to support clinical evaluation of BMN 307 being produced using a commercial scale manufacturing process to facilitate rapid clinical development and (iv) the possible approval and commercialization of BioMarin's product candidates, including vosoritide for the treatment of achondroplasia.

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 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, 2019 as such factors may be updated by any subsequent reports. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise.

BioMarin®, Brineura®, Firdapse®, Kuvan®, Naglazyme®, Palynziq® and Vimizim® are registered trademarks of BioMarin Pharmaceutical Inc., or its affiliates. Aldurazyme® is a registered trademark of BioMarin/Genzyme LLC.

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BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

June 30, 2019 and December 31, 2018

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

	June 30, 2019 (1)	December 31, 2018 (2)
	(unaudited)	
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 307,577	\$ 493,982
Short-term investments	423,526	590,326
Accounts receivable, net	377,150	342,633
Inventory	578,736	530,871
Other current assets	119,779	98,403
Total current assets	<u>1,806,768</u>	<u>2,056,215</u>
Noncurrent assets:		
Long-term investments	374,965	235,864
Property, plant and equipment, net	962,970	948,682
Intangible assets, net	476,632	491,808
Goodwill	197,039	197,039
Deferred tax assets	475,554	460,952
Other assets	99,456	36,568
Total assets	<u>\$ 4,393,384</u>	<u>\$ 4,427,128</u>
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 429,502	\$ 437,290
Short-term contingent consideration	9,926	85,951
Total current liabilities	<u>439,428</u>	<u>523,241</u>
Noncurrent liabilities:		
Long-term convertible debt, net	839,165	830,417
Long-term contingent consideration	50,151	46,883
Other long-term liabilities	103,686	58,647
Total liabilities	<u>1,432,430</u>	<u>1,459,188</u>
Stockholders' equity:		
Common stock, \$0.001 par value: 500,000,000 shares authorized; 179,433,316 and 178,252,954 shares issued and outstanding, respectively.	179	178
Additional paid-in capital	4,744,316	4,669,926
Company common stock held by Nonqualified Deferred Compensation Plan	(10,211)	(13,301)
Accumulated other comprehensive income	17,439	5,271
Accumulated deficit	<u>(1,790,769)</u>	<u>(1,694,134)</u>
Total stockholders' equity	<u>2,960,954</u>	<u>2,967,940</u>
Total liabilities and stockholders' equity	<u>\$ 4,393,384</u>	<u>\$ 4,427,128</u>

- As of January 1, 2019, the Company adopted the requirements of Accounting Standards Codification 842, *Leases*, using the modified retrospective method as of the effective date, and as a result, Other Assets and Liabilities are not comparable to the prior periods presented.
- December 31, 2018 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, 2018, filed with the U.S. Securities and Exchange Commission (SEC) on February 28, 2018.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
Three and Six Months Ended June 30, 2019 and 2018
(In thousands of U.S. dollars, except per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
REVENUES:				
Net product revenues	\$ 379,075	\$ 367,786	\$ 773,558	\$ 736,885
Royalty and other revenues	8,688	5,059	14,950	9,407
Total net revenues	<u>387,763</u>	<u>372,845</u>	<u>788,508</u>	<u>746,292</u>
OPERATING EXPENSES:				
Cost of sales	77,436	79,019	166,618	161,352
Research and development	185,641	175,582	369,232	359,530
Selling, general and administrative	160,754	153,280	322,912	291,616
Intangible asset amortization and contingent consideration	20,286	10,227	40,051	23,429
Gain on sale of intangible assets	(15,000)	(20,000)	(15,000)	(20,000)
Total operating expenses	<u>429,117</u>	<u>398,108</u>	<u>883,813</u>	<u>815,927</u>
LOSS FROM OPERATIONS	<u>(41,354)</u>	<u>(25,263)</u>	<u>(95,305)</u>	<u>(69,635)</u>
Equity in the loss of BioMarin/Genzyme LLC	(44)	(107)	(229)	(39)
Interest income	5,899	5,569	12,197	10,803
Interest expense	(6,866)	(12,225)	(13,593)	(23,787)
Other income, net	470	2,849	2,078	2,677
LOSS BEFORE INCOME TAXES	<u>(41,895)</u>	<u>(29,177)</u>	<u>(94,852)</u>	<u>(79,981)</u>
Benefit from income taxes	(4,460)	(12,385)	(944)	(19,040)
NET LOSS	<u>\$ (37,435)</u>	<u>\$ (16,792)</u>	<u>\$ (93,908)</u>	<u>\$ (60,941)</u>
NET LOSS PER SHARE, BASIC AND DILUTED	<u>\$ (0.21)</u>	<u>\$ (0.09)</u>	<u>\$ (0.53)</u>	<u>\$ (0.35)</u>
Weighted average common shares outstanding, basic and diluted	<u>179,048</u>	<u>176,873</u>	<u>178,662</u>	<u>176,405</u>

The following table presents Net Product Revenues by Product:

Net Product Revenues by Product
(In millions of U.S. dollars)
(unaudited)

	Three Months Ended			Six Months Ended		
	June 30,			June 30,		
	2019	2018	% Change	2019	2018	% Change
Brineura	\$ 14.8	\$ 10.9	36 %	\$ 27.0	\$ 17.8	52 %
Firdapse	5.5	5.2	6 %	10.6	10.1	5 %
Naglazyme	98.2	91.1	8 %	185.1	166.1	11 %
PKU franchise	132.1	109.0	21 %	251.3	208.1	21 %
Vimizim	122.7	127.6	(4) %	248.5	244.7	2 %
Net Product Revenues Marketed by BioMarin	<u>373.3</u>	<u>343.8</u>		<u>722.5</u>	<u>646.8</u>	
Aldurazyme Net Product Revenues Marketed by Genzyme	5.8	24.0	(76) %	51.1	90.1	(43) %
Total Net Product Revenues	<u>\$ 379.1</u>	<u>\$ 367.8</u>		<u>\$ 773.6</u>	<u>\$ 736.9</u>	

The following table presents Net Product Revenues for the PKU Franchise by Product:

Net Product Revenues by Product for the PKU Franchise
(In millions of U.S. dollars)
(unaudited)

	Three Months Ended			Six Months Ended		
	June 30,			June 30,		
	2019	2018	% Change	2019	2018	% Change
Kuvan	\$ 113.3	\$ 109.0	4 %	\$ 220.2	\$ 208.1	6 %
Palynziq	18.8	—	n/a	31.1	—	n/a
Total PKU franchise	<u>\$ 132.1</u>	<u>\$ 109.0</u>	21 %	<u>\$ 251.3</u>	<u>\$ 208.1</u>	21 %

The following table presents Genzyme's Aldurazyme Revenues:

Aldurazyme Revenues Provided by Genzyme to BioMarin
(In millions of U.S. dollars)
(unaudited)

Three Months Ended	Six Months Ended
June 30,	June 30,

	<u>2019</u>	<u>2018</u>	<u>% Change</u>	<u>2019</u>	<u>2018</u>	<u>% Change</u>
Genzyme's Aldurazyme Revenues	\$ 60.4	\$ 61.6	(2) %	\$ 136.1	\$ 124.5	9 %

Non-GAAP Information

The results presented in this press release include both GAAP information and Non-GAAP information. As used in this release, Non-GAAP Income 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 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 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 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 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)

	<u>Three Months Ended</u>		<u>Six Months Ended</u>		<u>Guidance</u>		
	<u>June 30,</u>		<u>June 30,</u>		<u>Year Ending</u>		
	<u>2019</u>	<u>2018</u>	<u>2019</u>	<u>2018</u>	<u>December 31, 2019</u>		
GAAP Net Loss	\$ (37.4)	\$ (16.8)	\$ (93.9)	\$ (60.9)	\$ (45.0)	—	\$ (85.0)
Interest expense, net	1.0	6.7	1.4	13.0	—	—	10.0
Benefit from income taxes	(4.5)	(12.3)	(0.9)	(19.0)	(50.0)	—	(30.0)
Depreciation expense	12.9	13.5	27.9	29.5	45.0	—	60.0
Amortization expense	13.4	7.5	20.9	15.1	40.0	—	55.0
Stock-based compensation expense	39.8	38.6	82.6	75.2	150.0	—	175.0
Contingent consideration expense	6.9	2.7	19.2	8.3	20.0	—	30.0
Gain on sale of intangible assets	(15.0)	(20.0)	(15.0)	(20.0)	(30.0)	—	(45.0)
Non-GAAP Income	<u>\$ 17.1</u>	<u>\$ 19.9</u>	<u>\$ 42.2</u>	<u>\$ 41.2</u>	<u>\$ 130.0</u>	<u>—</u>	<u>\$ 170.0</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)

	<u>Three months ended June 30,</u>							
	<u>2019</u>				<u>2018</u>			
	<u>Adjustments</u>				<u>Adjustments</u>			
	<u>GAAP</u>			<u>GAAP</u>				<u>Non-GAAP</u>
	<u>Reported</u>	<u>Amortization</u>	<u>Stock-Based Compensation, Contingent Consideration and Other Adjustments</u>	<u>Reported</u>	<u>Amortization</u>	<u>Stock-Based Compensation, Contingent Consideration and Other Adjustments</u>		<u>Reported</u>
Cost of sales	<u>\$ 77.4</u>	<u>\$ —</u>	<u>\$ (3.7)</u>	<u>\$ 73.7</u>	<u>\$ 79.1</u>	<u>\$ —</u>	<u>\$ (3.3)</u>	<u>\$ 75.8</u>
Research and development	<u>185.6</u>	<u>(7.3)</u>	<u>(14.9)</u>	<u>163.4</u>	<u>175.6</u>	<u>(7.9)</u>	<u>(15.5)</u>	<u>152.2</u>
Selling, general and administrative	<u>160.8</u>	<u>(5.6)</u>	<u>(21.2)</u>	<u>134.0</u>	<u>153.3</u>	<u>(5.6)</u>	<u>(19.8)</u>	<u>127.9</u>

Intangible asset amortization and contingent consideration	20.3	(13.4)	(6.9)	—	10.2	(7.5)	(2.7)	—
Gain on sale of intangible assets	(15.0)	—	15.0	—	(20.0)	—	20.0	—
Interest expense, net	(1.0)	1.0	—	—	(6.7)	6.7	—	—
Benefit from income taxes	(4.5)	4.5	—	—	(12.3)	12.3	—	—
GAAP Net Loss/Non-GAAP Income (Loss)	(37.4)	22.8	31.7	17.1	(16.8)	15.4	21.3	19.9

Six months ended June 30,

	2019				2018			
	Adjustments				Adjustments			
	GAAP Reported	Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non-GAAP	GAAP Reported	Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non-GAAP
Cost of sales	\$ 166.6	\$ —	\$ (8.5)	\$ 158.1	\$ 161.4	\$ —	\$ (6.4)	\$ 155.0
Research and development	369.2	(16.7)	(28.8)	323.7	359.5	(18.4)	(28.8)	312.3
Selling, general and administrative	322.9	(11.2)	(45.3)	266.4	291.6	(11.1)	(40.0)	240.5
Intangible asset amortization and contingent consideration	40.1	(20.9)	(19.2)	—	23.4	(15.1)	(8.3)	—
Gain on sale of intangible assets	(15.0)	—	15.0	—	(20.0)	—	20.0	—
Interest expense, net	(1.4)	1.4	—	—	(13.0)	13.0	—	—
Benefit from income taxes	(0.9)	0.9	—	—	(19.0)	19.0	—	—
GAAP Net Loss/Non-GAAP Income (Loss)	(93.9)	49.3	86.8	42.2	(60.9)	38.6	63.5	41.2

SOURCE BioMarin Pharmaceutical Inc.

<https://investors.biomin.com/2019-08-01-BioMarin-Announces-Second-Quarter-2019-Financial-Results>