

## BioMarin Announces Third Quarter 2019 Financial Results

- The Company Achieved Record Total Revenues of \$461.1 million; Increased 18% Year Over Year
- Full-year GAAP Loss and Non-GAAP Income Guidance Improved to Top-end of Range Driven by R&D Expense Management
- European Medicines Agency Granted Request for Accelerated Assessment of Valoctocogene Roxaparvovec
- Clinical Trial Application Submitted for BMN 307 Gene Therapy Product Candidate for Phenylketonuria
- BioMarin and Allievex Enter Into Licensing Agreement for Tralesinidase Alfa for Sanfilippo Syndrome Type B

SAN RAFAEL, Calif., Oct. 23, 2019 /PRNewswire/ --



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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2019	2018	% Change	2019	2018	% Change
Total Revenues	\$ 461.1	\$ 391.7	18 %	\$ 1,249.6	\$ 1,138.0	10 %
Net Product Revenues Marketed by BioMarin (1)	428.1	358.7	19 %	1,150.6	1,005.5	14 %
Vimizim Net Product Revenues	163.5	123.3	33 %	412.0	368.0	12 %
Kuvan Net Product Revenues	120.6	113.3	6 %	340.8	321.4	6 %
Naglazyme Net Product Revenues	94.4	103.1	(8) %	279.5	269.2	4 %
Palynziq Net Product Revenues	24.1	4.1	N/M	55.2	4.1	N/M
Brineura Net Product Revenues	19.8	9.9	100 %	46.8	27.7	69 %
Aldurazyme Net Product Revenues	22.8	27.6	(17) %	73.9	117.7	(37) %
GAAP Net Income (Loss)	\$ 55.0	\$ (12.6)		\$ (38.9)	\$ (73.6)	
GAAP Net Income (Loss) per Share - Basic	\$ 0.31	\$ (0.07)		\$ (0.22)	\$ (0.42)	
GAAP Net Income (Loss) per Share - Diluted	\$ 0.30	\$ (0.07)		\$ (0.22)	\$ (0.42)	
Non-GAAP Income (2)	\$ 78.1	\$ 60.7		\$ 120.1	\$ 101.8	

	September 30, 2019	December 31, 2018
Cash, cash equivalents and investments	\$1,152.6	\$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 9 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 10 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 third quarter ended September 30, 2019.

Total Net Product Revenues for the third of quarter 2019 increased to \$450.9 million, compared to \$386.3 million for the third quarter of 2018. The increase in Net Product Revenues was attributed to the following:

- Vimizim Net Product Revenues increased by \$40.2 million, or 33%, driven primarily by increased sales volume driven by government orders in certain Middle Eastern countries, a large order from Brazil as well as smaller orders from other Latin American countries;
- Palynziq Net Product Revenues increased by \$20.0 million, driven by a combination of revenue from patients achieving maintenance dosing and new patients initiating therapy in the U.S. Palynziq received approval from the U.S. Food and Drug Administration (FDA) in May 2018 and launched in the third quarter of that year. Palynziq received European Medicines Agency (EMA) approval in May 2019 and commercial sales in Europe are expected to commence in the fourth quarter of 2019;
- Brineura Net Product Revenues increased by \$9.9 million, or 100%, due in large part by growth in the number of patients across all regions; and
- Kuvan Net Product Revenues increased by \$7.3 million, or 6%, primarily driven by an increase in the number of patients in North America; partially offset by
- Naglazyme Net Product Revenues decreased by \$8.7 million, or 8%, primarily due to decreased sales volume driven by government ordering patterns from certain Latin American and European countries; and
- Aldurazyme Net Product Revenues decreased \$4.8 million, due to the timing of customer acceptance for product shipped to Genzyme in the third quarter for which no revenue was recognized as of September 30, 2019.

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

- increased gross profits of \$51.3 million driven by increased product sales;
- increased tax benefit, which is primarily attributed to quarterly fluctuations in the mix and timing of our profits and losses on a territorial basis and reversals of certain tax reserves that were no longer required, partially offset by
- higher selling, general and administrative (SG&A) expense related to pre-commercialization activities for valoctocogene roxaparvovec, support of the EU commercial launch and continued U.S. expansion of Palynziq, and increased general and administrative expense primarily attributed to personnel-related costs resulting from increased headcount to support our growth; and
- higher research and development (R&D) expense related to preclinical activities for BMN 307 and clinical activities for the Company's vosoritide and valoctocogene roxaparvovec development programs, partially 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 roxaparvovec development program and subsequent activities implemented to pursue an expedited regulatory path forward.

GAAP Net Income (Loss) Guidance for the full-year 2019 was narrowed to the low-end of the previously reported range to a loss of between \$45 million to \$65 million, reflecting continued expense management and strong net product sales.

Non-GAAP Income for the third quarter of 2019 increased \$17.4 million, or 29%, to \$78.1 million, compared to \$60.7 million for the same period in 2018. The increase in Non-GAAP Income for the quarter, compared to the same period in 2018, was attributed to higher gross profit and driven by R&D expense management, partially offset by higher SG&A expense. The increase in Non-GAAP Income for the third quarter of 2019 resulted in narrowed full-year Non-GAAP income guidance to the high-end of the previously reported the range to between \$150 million and \$170 million.

As of September 30, 2019, BioMarin had cash, cash equivalents and investments totaling approximately \$1.2 billion, as compared to \$1.3 billion on December 31, 2018.

Commenting on third quarter results, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "BioMarin is entering a stage that I believe will bring significant growth as we get closer to submitting marketing applications for valoctocogene roxaparvovec for severe hemophilia A and a pivotal data read-out for vosoritide for children with achondroplasia. These potential new products, combined with our strong base business and continued financial discipline, position us for significant growth and expansion beginning in the very near future. Later this quarter, both the U.S. and European marketing applications for valoctocogene roxaparvovec for severe hemophilia A will be submitted. If the applications are approved, we could potentially be launching the first approved gene therapy product in hemophilia A in the second half of 2020. We are also encouraged by the recent recommendation by the European Medicines Agency (EMA) to grant our request for accelerated assessment of valoctocogene roxaparvovec for severe hemophilia A. We are very pleased with the level of engagement we have had with global health authorities, as it aligns with our belief that gene therapy will be the next wave of innovation for treating people with severe hemophilia A."

Mr. Bienaimé continued, "After valoctocogene roxaparvovec, vosoritide for the treatment of children with achondroplasia is our next significant commercial opportunity. Phase 3 results with vosoritide are expected by year-end. Our most recently initiated study with vosoritide, a global Phase 2 in infants and young children (less than 60 months old), is rounding the corner to complete enrollment. All subjects from ages 2 through 5 years have been enrolled. The second cohort, which includes children ages 6 months through 2 years old will complete by year-end, and the youngest cohort, infants up to 6 months old, has recently started enrolling. Interest in this study with very young children has been extremely robust, demonstrating that families are interested in treatment for their children as early as possible. Our most recently approved product, Palynziq for the treatment of phenylketonuria, is on a very strong launch trajectory since approval last year in the U.S. We have been very pleased with the pace of the U.S. launch, as we ended the third quarter with 670 patients on reimbursed Palynziq, and an additional 153 naïve patients having completed enrollment and awaiting their first injection. Building on this success, and as part of our strategy to further build our presence in the PKU market, we submitted the clinical trial application (CTA) for BMN 307, our gene therapy product for PKU, in September. 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 (in millions, except %)**

Item	2019 Guidance, provided February 21, 2019		2019 Updated Guidance, provided October 23, 2019	
Total Revenues	\$ 1,680	to \$ 1,750	\$ 1,690	to \$ 1,720
Vimizim Net Product Revenues	\$ 530	to \$ 570	\$ 540	to \$ 570
Kuvan Net Product Revenues	\$ 420	to \$ 460	\$ 455	to \$ 475
Naglazyme Net Product Revenues	\$ 350	to \$ 380	\$ 360	to \$ 380
Palynziq Net Product Revenues	\$ 70	to \$ 100	\$ 80	to \$ 100
Brineura Net Product Revenues	\$ 55	to \$ 75		Unchanged
Cost of Sales (% of Total Revenues)	20 %	to 21 %		Unchanged
Research and Development Expense	\$ 740	to \$ 780	\$ 710	to \$ 740
Selling, General and Administrative Expense	\$ 650	to \$ 690	\$ 670	to \$ 690
GAAP Net Loss	\$ (45)	to \$ (85)	\$ (45)	to \$ (65)
Non-GAAP Income *	\$ 130	to \$ 170	\$ 150	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 10 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 roxaparvovec gene therapy for hemophilia A:** The European Medicines Agency (EMA) recently granted BioMarin's request for accelerated assessment of valoctocogene roxaparvovec, for adults with severe hemophilia A. Accelerated assessment reduces the time-frame for the EMA Committee for Medicinal Products for Human Use (CHMP) and Committee for Advanced Therapies (CAT) to review a Marketing Authorization Application (MAA) for an Advanced Therapy Medicinal Product (ATMP). Applications are eligible for accelerated assessment if the CHMP and CAT decide the product is of major interest for public health, particularly from the point of view of therapeutic innovation. Evaluating a MAA under the EMA centralized procedure can take up to 210 days, not counting clock stops when applicants are requested to provide additional information. On request, the CHMP and CAT can reduce the time-frame to 150 days if the applicant provides sufficient justification for an accelerated assessment. The decision to grant accelerated assessment has no impact on the eventual CHMP and CAT opinion on whether a marketing authorization should be granted.

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 EMA in the fourth quarter of 2019 for valoctocogene roxaparvovec with the 6e13 vg/kg dose. The submissions will be based on the recently completed Phase 3 interim analysis and the updated three-year Phase 1/2 data of patients treated with valoctocogene roxaparvovec. 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.

Enrollment in the GENE8-1 Phase 3 study is expected to be complete by R&D Day with 52-week results from the 130 subjects expected at the end

of 2020. Although the trial is open label, BioMarin has implemented a data access plan designed to significantly mirror a blinded trial. This restricts the release of any ongoing data to a small group of medical personnel monitoring and managing the trial, and then, only to the extent necessary to perform their monitoring responsibilities.

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.

BioMarin intends to provide a 4 year update with the 6e13 vg/kg dose subjects and a 3 year update with the 4e13 vg/kg dose subjects from the ongoing Phase 2 study in mid-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 September 30, 2019, 670 patients were on reimbursed Palynziq, with an additional 153 naïve patients enrolled and awaiting their first treatment with commercial Palynziq. Of the 670 patients on therapy at the end of the third quarter, 528 were formerly naïve patients and 142 had transitioned from clinical studies. Of the 125 PKU clinics in the U.S., 97 unique clinics had at least one complete patient enrollment in the REMS program as of September 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. The Company is in the process of securing reimbursement on a country-by-country basis across the European Union and anticipates meaningful revenue contributions from this region in 2020.

- **Vosoritide for children with achondroplasia:** 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 is a randomized, double-blind placebo-controlled study of vosoritide in approximately 110 children with achondroplasia between the ages of 5 to 14 years. Data from this study is expected by year-end 2019.

The Company plans to share 54-month results from the ongoing Phase 2 study with vosoritide in children ages 5 to 14 years at the upcoming R&D Day planned for November 14. These data are expected to corroborate maintenance of effect at the time of anticipated marketing application submissions.

The fourth component of the Company's global development program with vosoritide, includes a large Phase 2 study 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 at various stages of enrollment 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 will complete enrollment by year-end. Cohort 3, includes children ages 0 to 6 months old and began enrolling earlier this month.

- **BMN 307 gene therapy product candidate for phenylketonuria (PKU):** On October 21, 2019, BioMarin was granted Orphan drug designation from the FDA for BMN 307 for the treatment of phenylketonuria. On September 26, 2019, the Company submitted a CTA with the medicines and Healthcare Products Regulatory Agency (MHRA) in the U.K.

Preclinical data with BMN 307 demonstrated a lifetime Phe correction sustained at 80 weeks in 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 is being produced at BioMarin's gene therapy manufacturing facility, where valoctocogene roxaparvec is currently made, using a commercial scale manufacturing process to facilitate rapid clinical development.

- **Tralesinidase alfa (formerly referred to as BMN 250) for Mucopolysaccharidosis IIIB (MPS IIIB) or Sanfilippo Syndrome, Type B:** On October 23, 2019, the Company announced that it had entered into a licensing agreement with Allievox Corp. (Allievox) for tralesinidase alfa, an investigational Enzyme Replacement Therapy (ERT) for MPS IIIB or Sanfilippo Syndrome Type B. Under the terms of the agreement, Allievox will receive a worldwide, exclusive license to tralesinidase alfa. BioMarin is entitled to receive a minority equity stake in Allievox, milestone payments if certain development, regulatory and sales milestones are met by Allievox and royalties on net sales of tralesinidase alfa. Upon closing of the transaction, Allievox will assume all financial obligations associated with the development and commercialization of tralesinidase alfa other than certain continued manufacturing activities that will be paid by BioMarin. BioMarin will transfer all tralesinidase alfa-related clinical and regulatory activity and responsibilities to Allievox during a transition period following closing of the transaction. Tralesinidase alfa is currently being evaluated in ongoing natural history and clinical trials.
- **R&D Day to be held in New York November 14, 2019:** The Company plans to hold an investor event to discuss potential new product candidates, vosoritide, valoctocogene roxaparvec status and other general corporate updates. Please email [emily.white@bmrn.com](mailto:emily.white@bmrn.com) for more information.

BioMarin will host a conference call and webcast to discuss third quarter 2019 financial results today, Wednesday, October 23, 2019 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: 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](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, Cost of Sales, GAAP Net Loss, Non-GAAP Income and other specified income statement guidance for the full-year 2019; the financial performance of BioMarin as a whole; BioMarin's potential for significant growth and expansion; BioMarin anticipating significant milestones over the coming months; BioMarin's entitlement to receive of milestone payments and royalties from Allievox that licensed rights from BioMarin for tralesinidase alfa and Allievox's assumption of financial obligations related to the development and commercialization of tralesinidase alfa; 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 roxaparvec in both the U.S. and Europe, (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 roxaparvec and global

Phase 2 study with vosoritide, and (iv) announcements of data from those studies and trials, including BioMarin's Phase 3 program with valoctocogene roxaparvovec and the global Phase 3 study of vosoritide; the clinical development and commercialization of BioMarin's product candidates and commercial products, including (i) BioMarin's planned submission of marketing authorization applications for valoctocogene roxaparvovec, which if approved could potentially be the first approved gene therapy product for hemophilia A, (ii) product to support clinical evaluation of BMN 307 being produced using a commercial scale manufacturing process to facilitate rapid clinical development, (iii) the possible approval and commercialization of BioMarin's product candidates, including vosoritide for the treatment of achondroplasia, and (iv) the Company's securing of reimbursement for Palyzniq on a country-by-country basis in the EU and Palyzniq's revenue from the EU in 2020.

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; results and timing of current and planned preclinical studies and clinical trials and the release of data from those 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 June 30, 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®, Palyzniq® 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**

**September 30, 2019 and December 31, 2018**

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

	<b>September 30, 2019 (1)</b>	<b>December 31, 2018 (2)</b>
	(unaudited)	
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 423,220	\$ 493,982
Short-term investments	297,572	590,326
Accounts receivable, net	402,970	342,633
Inventory	609,049	530,871
Other current assets	126,548	98,403
Total current assets	<u>1,859,359</u>	<u>2,056,215</u>
Noncurrent assets:		
Long-term investments	431,804	235,864
Property, plant and equipment, net	969,300	948,682
Intangible assets, net	462,849	491,808
Goodwill	197,039	197,039
Deferred tax assets	525,131	460,952
Other assets	112,646	36,568
Total assets	<u>\$ 4,558,128</u>	<u>\$ 4,427,128</u>
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 483,745	\$ 437,290
Short-term contingent consideration	10,000	85,951
Total current liabilities	<u>493,745</u>	<u>523,241</u>
Noncurrent liabilities:		
Long-term convertible debt, net	843,616	830,417
Long-term contingent consideration	48,930	46,883
Other long-term liabilities	97,432	58,647
Total liabilities	<u>1,483,723</u>	<u>1,459,188</u>
Stockholders' equity:		
Common stock, \$0.001 par value: 500,000,000 shares authorized; 179,604,381 and 178,252,954 shares issued and outstanding, respectively.	180	178
Additional paid-in capital	4,782,916	4,669,926
Company common stock held by Nonqualified Deferred Compensation Plan	(9,961)	(13,301)
Accumulated other comprehensive income	37,003	5,271
Accumulated deficit	(1,735,733)	(1,694,134)
Total stockholders' equity	<u>3,074,405</u>	<u>2,967,940</u>
Total liabilities and stockholders' equity	<u>\$ 4,558,128</u>	<u>\$ 4,427,128</u>

(1) 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.

(2) 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 Nine Months Ended September 30, 2019 and 2018**  
(In thousands of U.S. dollars, except per share amounts)  
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
<b>REVENUES:</b>				
Net product revenues	\$ 450,900	\$ 386,320	\$ 1,224,458	\$ 1,123,205
Royalty and other revenues	10,197	5,386	25,147	14,793
Total net revenues	<u>461,097</u>	<u>391,706</u>	<u>1,249,605</u>	<u>1,137,998</u>
<b>OPERATING EXPENSES:</b>				
Cost of sales	\$ 96,949	\$ 78,893	\$ 263,567	\$ 240,245
Research and development	172,963	161,408	542,195	520,938
Selling, general and administrative	170,112	148,566	493,024	440,182
Intangible asset amortization and contingent consideration	17,063	18,580	57,114	42,009
Gain on sale of intangible assets	—	—	(15,000)	(20,000)
Total operating expenses	<u>\$ 457,087</u>	<u>\$ 407,447</u>	<u>\$ 1,340,900</u>	<u>\$ 1,223,374</u>
<b>INCOME (LOSS) FROM OPERATIONS</b>	<u>\$ 4,010</u>	<u>\$ (15,741)</u>	<u>\$ (91,295)</u>	<u>\$ (85,376)</u>
Equity in the loss of BioMarin/Genzyme LLC	(551)	(468)	(780)	(507)
Interest income	5,340	6,338	17,537	17,141
Interest expense	(2,937)	(12,131)	(16,530)	(35,918)
Other income, net	3,960	2,589	6,038	5,266
<b>INCOME (LOSS) BEFORE INCOME TAXES</b>	<u>9,822</u>	<u>(19,413)</u>	<u>(85,030)</u>	<u>(99,394)</u>
Benefit from income taxes	(45,214)	(6,793)	(46,158)	(25,833)
<b>NET INCOME (LOSS)</b>	<u>\$ 55,036</u>	<u>\$ (12,620)</u>	<u>\$ (38,872)</u>	<u>\$ (73,561)</u>
<b>NET INCOME (LOSS) PER SHARE, BASIC</b>	<u>\$ 0.31</u>	<u>\$ (0.07)</u>	<u>\$ (0.22)</u>	<u>\$ (0.42)</u>
<b>NET LOSS PER SHARE, DILUTED</b>	<u>\$ 0.30</u>	<u>\$ (0.07)</u>	<u>\$ (0.22)</u>	<u>\$ (0.42)</u>
Weighted average common shares outstanding, basic	179,289	177,481	178,873	176,767
Weighted average common shares outstanding, diluted	<u>185,924</u>	<u>177,481</u>	<u>178,873</u>	<u>176,767</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 September 30,			Nine Months Ended September 30,		
	2019	2018	% Change	2019	2018	% Change
Brineura	\$ 19.8	\$ 9.9	100 %	\$ 46.8	\$ 27.7	69 %
Firdapse	5.7	5.0	14 %	16.3	15.1	8 %
Naglazyme	94.4	103.1	(8) %	279.5	269.2	4 %
PKU franchise	144.7	117.4	23 %	396.0	325.5	22 %
Vimizim	163.5	123.3	33 %	412.0	368.0	12 %
Net Product Revenues Marketed by BioMarin	428.1	358.7		1,150.6	1,005.5	
Aldurazyme Net Product Revenues Marketed by Genzyme	22.8	27.6	(17) %	73.9	117.7	(37) %
Total Net Product Revenues	<u>\$ 450.9</u>	<u>\$ 386.3</u>		<u>\$ 1,224.5</u>	<u>\$ 1,123.2</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 September 30,			Nine Months Ended September 30,		
	2019	2018	% Change	2019	2018	% Change
Kuvan	\$ 120.6	\$ 113.3	6 %	\$ 340.8	\$ 321.4	6 %
Palynziq	24.1	4.1	N/M	55.2	4.1	N/M
Total PKU franchise	<u>\$ 144.7</u>	<u>\$ 117.4</u>	23 %	<u>\$ 396.0</u>	<u>\$ 325.5</u>	22 %

**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 Income/Loss excluding net interest expense, provision for (benefit from) income taxes, depreciation expense, amortization expense, stock-based compensation expense, contingent consideration expense and, in certain periods, certain other specified items, as detailed below when applicable. In addition, BioMarin includes in this press release the effects of these adjustments on certain components of GAAP Net Income/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 Income (Loss) to Non-GAAP Income:

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

GAAP Net Income (Loss)	Three Months Ended September 30,		Nine Months Ended September 30,		Guidance Year Ending		
	2019	2018	2019	2018	December 31, 2019		
	\$ 55.0	\$ (12.6)	\$ (38.9)	\$ (73.6)	\$ (45.0)	— \$ (65.0)	
Interest expense, net	(2.4)	5.8	(1.0)	18.8	—	—	10.0
Benefit from income taxes	(45.2)	(6.8)	(46.2)	(25.8)	(30.0)	—	(50.0)
Depreciation expense	14.4	18.6	42.3	48.1	45.0	—	60.0
Amortization expense	16.3	7.6	37.2	22.7	40.0	—	55.0
Stock-based compensation expense	39.2	37.1	121.8	112.3	150.0	—	175.0
Contingent consideration expense	0.8	11.0	19.9	19.3	20.0	—	30.0
Gain on sale of intangible assets	—	—	(15.0)	(20.0)	(30.0)	—	(45.0)
Non-GAAP Income	\$ 78.1	\$ 60.7	\$ 120.1	\$ 101.8	\$ 150.0	—	\$ 170.0

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 September 30,**

	2019				2018			
	Adjustments			Non-GAAP	Adjustments			Non-GAAP
	GAAP Reported	Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments		GAAP Reported	Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	
Cost of sales	\$ 96.9	\$ —	\$ (4.1)	\$ 92.8	\$ 78.9	\$ —	\$ (4.0)	\$ 74.9
Research and development	173.0	(6.4)	(14.3)	152.3	161.4	(7.0)	(14.3)	140.1
Selling, general and administrative	170.1	(8.0)	(20.8)	141.3	148.6	(11.6)	(18.8)	118.2
Intangible asset amortization and contingent consideration	17.1	(16.3)	(0.8)	—	18.6	(7.6)	(11.0)	—
Gain on sale of intangible assets	—	—	—	—	—	—	—	—
Interest expense, net	2.4	(2.4)	—	—	(5.8)	5.8	—	—
Benefit from income taxes	(45.2)	45.2	—	—	(6.8)	6.8	—	—
GAAP Net Income (Loss)/Non-GAAP Income	55.0	(16.9)	40.0	78.1	(12.6)	25.2	48.1	60.7

**Nine months ended September 30,**

	2019				2018			
	Adjustments			Non-GAAP	Adjustments			Non-GAAP
	GAAP Reported	Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments		GAAP Reported	Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	
Cost of sales	\$ 263.6	\$ —	\$ (12.6)	\$ 251.0	\$ 240.2	\$ —	\$ (10.4)	\$ 229.8
Research and development	542.2	(23.1)	(43.1)	476.0	520.9	(25.4)	(43.1)	452.4
Selling, general and administrative	493.0	(19.2)	(66.1)	407.7	440.2	(22.7)	(58.8)	358.7

Intangible asset amortization and contingent consideration	<b>57.1</b>	(37.2)	(19.9)	—	<b>42.0</b>	(22.7)	(19.3)	—
Gain on sale of intangible assets	<b>(15.0)</b>	—	15.0	—	<b>(20.0)</b>	—	20.0	—
Interest expense, net	<b>1.0</b>	(1.0)	—	—	<b>(18.8)</b>	18.8	—	—
Benefit from income taxes	<b>(46.2)</b>	46.2	—	—	<b>(25.8)</b>	25.8	—	—
GAAP Net Loss/Non-GAAP Income	<b>(38.9)</b>	32.3	126.7	120.1	<b>(73.6)</b>	63.8	111.6	101.8

SOURCE BioMarin Pharmaceutical Inc.

<https://investors.biomin.com/2019-10-23-BioMarin-Announces-Third-Quarter-2019-Financial-Results>