

**BioMarin Announces Third Quarter 2020 Total Revenues of \$477 Million**

**- Top-line Results from 52-week Phase 3 Study with Valoctocogene Roxaparvovec for Severe Hemophilia A Expected Early 2021**

**- Submission of Phase 3 Results from 134 Subjects with 1 to 2 Years of Data with Valoctocogene Roxaparvovec for Severe Hemophilia A to the European Medicines Agency (EMA) Expected in the Second Quarter of 2021**

**- Marketing Applications for Vosoritide to Treat Children with Achondroplasia under Review in Europe and the U.S.; Committee for Medicinal Products for Human Use (CHMP) Opinion Expected in the Second Half of 2021 and U.S. Prescription Drug User Fee Act (PDUFA) Target Action Date Expected August 20, 2021**

**- 2020 GAAP Net Income Guidance Reflects Impact of Tax Benefit; Reduced Operating Expenses Drive Improved 2020 Non-GAAP Income Guidance; 2020 Full-year Total Revenue Guidance Revised due to Impact of the Coronavirus (COVID-19) Pandemic and Absence of Valoctocogene Roxaparvovec Revenue Contributions in 2020**

SAN RAFAEL, Calif., Nov. 5, 2020 /PRNewswire/ --

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2020	2019	% Change	2020	2019	% Change
Total Revenues	\$ 476.8	\$ 461.1	3 %	\$ 1,408.3	\$ 1,249.6	13 %
Net Product Revenues Marketed by BioMarin (1)	419.8	428.1	(2) %	1,239.9	1,150.6	8 %
Vimizim Net Product Revenues	147.9	163.5	(10) %	401.8	412.0	(2) %
Kuvan Net Product Revenues	124.1	120.6	3 %	368.7	340.8	8 %
Naglazyme Net Product Revenues	76.3	94.4	(19) %	271.6	279.5	(3) %
Palynziq Net Product Revenues	46.1	24.1	91 %	121.4	55.2	120 %
Brineura Net Product Revenues	25.4	19.8	28 %	75.2	46.8	61 %
Aldurazyme Net Product Revenues	40.9	22.8	79 %	128.9	73.9	74 %
GAAP Net Income (Loss)	\$ 784.8	\$ 55.0		\$ 837.0	\$ (38.9)	
GAAP Net Income (Loss) per Share – Basic	\$ 4.33	\$ 0.31		\$ 4.63	\$ (0.22)	
GAAP Net Income (Loss) per Share – Diluted	\$ 4.01	\$ 0.30		\$ 4.39	\$ (0.22)	
Non-GAAP Income (2)	\$ 98.7	\$ 78.1		\$ 272.6	\$ 120.1	

	September 30, 2020	December 31, 2019
Cash, cash equivalents and investments	\$ 1,770.8	\$ 1,165.8

(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. In January 2020, BioMarin divested the Firdapse assets to a third party in a sale transaction. The sale is reflected in the Company's consolidated financial statements for the three and nine months ending September 30, 2020; as a result of the transaction BioMarin will not recognize Net Product Revenues from Firdapse in the future.

(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 third quarter ended September 30, 2020.

Net Product Revenues for the third quarter of 2020 increased to \$460.7 million, compared to \$450.9 million in the third quarter of 2019. The increase in Net Product Revenues was primarily attributed to the following:

- Palynziq Net Product Revenues increased by \$22.0 million driven by a combination of revenue from U.S. patients achieving maintenance dosing and new patients initiating therapy;
- Aldurazyme Net Product Revenues increased by \$18.1 million due to higher sales volume to Genzyme;
- Naglazyme and Vimizim Net Product Revenues decreased by an aggregate of \$33.7 million primarily due to timing of orders placed from Latin America as well as the impact of missed infusions resulting from the COVID-19 pandemic.

The increase in GAAP Net Income for the third quarter of 2020, compared to the same period in 2019 was primarily due to the following:

- an increase in the benefit from income taxes of \$800.8 million primarily due the completion of an intra-entity transfer of certain intellectual property (IP) rights to an Irish subsidiary where the Company's Ex-US regional headquarters are located and has significant manufacturing and commercial operations, to better align ownership of IP rights with how the business operates resulting in a tax benefit of \$835.1 million based on the fair value of the transferred IP rights; and
- decreased research and development (R&D) expense primarily resulting from decreased clinical manufacturing costs for BMN 307 and lower clinical activity spend for valoctocogene roxaparvovec gene therapy programs; partially offset by
- an increase in Cost of Sales of \$91.8 million primarily attributed to the \$81.2 million reserve of valoctocogene roxaparvovec pre-launch inventory due to delays in anticipated regulatory approvals; and
- higher selling, general and administrative (SG&A) expense related to pre-commercialization activities for valoctocogene roxaparvovec.

Non-GAAP Income for the third quarter of 2020 increased to \$98.7 million, compared to Non-GAAP Income of \$78.1 million for the same period in 2019. The increase in Non-GAAP Income for the quarter, compared to the same period in 2019, was attributed to decreased R&D expense and higher gross profit, excluding the \$81.2 million pre-launch inventory charge, partially offset by higher SG&A expense.

As of September 30, 2020, BioMarin had cash, cash equivalents and investments totaling approximately \$1.8 billion, which includes net proceeds of \$535.8 million from the Company's May 2020 convertible debt offering, as compared to \$1.2 billion on December 31, 2019. On October 15, 2020, the Company's 1.50% senior subordinate convertible notes matured and were settled in cash for approximately \$375.0 million.

Commenting on third quarter 2020 results, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "While the impact of COVID-19 continued through the third quarter, BioMarin employees remained focused on our mission to serve patients and ensure the steady supply of our critically-important medicines. In these continued unpredictable times, the essential nature of our products to the people who rely on them remains constant."

Mr. Bienaimé continued, "In the third quarter we received unexpected news on the status of our application with valoctocogene roxaparvovec for hemophilia A from health authorities, which resulted in a delay in potential approval timelines. However, we remain confident in our valoctocogene roxaparvovec gene therapy and its potential to redefine the treatment paradigm for people with hemophilia A. We continue to work with the health authorities to align on next steps toward approval. Our 134-subject Phase 3 study with valoctocogene roxaparvovec will complete one-year of observation in all subjects later this month, and we anticipate sharing top-line results comprising 1 to 2 years of follow-up from that study, in the first quarter of 2021. We also plan to submit the complete one-year Phase 3 data to the EMA in the second quarter of 2021."

"Vosoritide for the treatment of achondroplasia is advancing as planned with applications under review in both the U.S. and Europe with potential regulatory approvals anticipated in 2021. The significant unmet medical need for children with achondroplasia has enabled BioMarin to build a multi-pronged dossier of clinical studies. It includes the highly statistically significant placebo-controlled Phase 3 results, long-term clinical results in 5 to 18 year-olds from our Phase 2 study, natural history data, and the ongoing study of newborns through 5 years, which is nearing enrollment completion. The positive results from our vosoritide clinical programs bolster our confidence in the potential for this drug to be the first pharmacological treatment to address the underlying cause of achondroplasia. Interest in our clinical studies with vosoritide has been extremely robust, demonstrating that many families are keen to seek early treatment for their children."

Mr. Bienaimé concluded, "Despite the impact from COVID-19 and the timing set-back on the potential approval of valoctocogene roxaparvovec, we remain confident in our business. BioMarin fundamentals are strong, driven by our global base business of essential medicines and cash position, but our people and pursuit and development of innovative therapies will always be our most important assets."

#### **2020 Full-Year Financial Guidance (in millions, except %)**

<b>Item</b>	<b>Provided August 4, 2020</b>		<b>Revised November 5, 2020</b>	
Total Revenues <sup>(1)</sup>	\$1,850	to	\$1,950	\$1,810 to \$1,870
Vimizim Net Product Revenues	\$530	to	\$570	\$515 to \$545

Kuvan Net Product Revenues	\$430	to	\$480	Unchanged	
Naglazyme Net Product Revenues	\$360	to	\$400	\$370	to \$400
Palyngiq Net Product Revenues	\$160	to	\$190	Unchanged	
Brineura Net Product Revenues	\$85	to	\$115	\$90	to \$110
Cost of Sales (% of Total Revenues) <sup>(2)</sup>	20%	to	21%	26%	to 28%
Research and Development Expense	\$675	to	\$725	\$630	to \$670
Selling, General and Administrative Expense	\$780	to	\$830	\$725	to \$765
GAAP Net Income	\$720	to	\$980	\$760	to \$820
Non-GAAP Income <sup>(3)</sup>	\$260	to	\$310	\$280	to \$330

- (1) Revenue guidance reflects BioMarin's projected impact of the COVID-19 pandemic on its global revenue sources, mostly in the form of demand interruptions such as missed patient infusions and delayed treatment starts for new patients. Total Revenue guidance also reflects the impact of the valoctocogene roxaparvovec FDA Complete Response Letter whereby the Company no longer expects any revenue from valoctocogene roxaparvovec in 2020 and the previously anticipated October 2020 loss of market exclusivity for Kuvan in the U.S. Management also notes that the impact of COVID-19 on revenues is expected to persist into 2021 due primarily to the effect of delays in new patients initiating therapy.
- (2) Revised Cost of Sales guidance for 2020 reflects the incremental charge of \$81.2 million during the third quarter of 2020 related to valoctocogene roxaparvovec pre-launch inventory reserves.
- (3) 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 roxaparvovec gene therapy for severe hemophilia A:** BioMarin is working with the U.S. Food and Drug Administration to align on steps forward to obtain marketing approval following the August 18, 2020 Complete Response Letter to the Company's Biologics License Application for valoctocogene roxaparvovec gene therapy for severe hemophilia A. The FDA recommended that the Company complete the Phase 3 study and submit two-year follow-up safety and efficacy data on all study participants. The Phase 3 study was fully enrolled in November 2019 and will complete one-year of follow-up of all patients in November 2020. The Company intends to share the one-year top-line Phase 3 data in the first quarter of 2021.

Additionally, the EMA recently requested the 52-week results from the full Phase 3 study cohort of 134 subjects to inform their benefit-risk assessment. To facilitate this submission within the EMA regulatory framework, BioMarin recently withdrew the MAA and plans to resubmit the MAA with these data to the EMA in the second quarter of 2021.

- **Vosoritide for children with achondroplasia:** Marketing applications for vosoritide were recently validated and accepted, by EMA and FDA, respectively. The CHMP opinion is expected in Europe in the second half of 2021. The U.S. New Drug Application for vosoritide is under Standard review by the FDA with a Prescription Drug User Fee Act target action date of August 20, 2021. Vosoritide is an investigational, once daily injection of an analog of C-type Natriuretic Peptide. If approved, vosoritide would be the only therapeutic treatment available for children with achondroplasia.
- **Palyngiq for Phenylketonuria (PKU):** On October 7, 2020 the Company announced that the FDA approved the supplemental Biologics License Application (sBLA) to increase the maximum allowable dose of Palyngiq (pegvaliase-pqgz) Injection for treatment of adults with Phenylketonuria (PKU) to 60 mg daily. Previously, the maximum dose was 40 mg daily. In the Phase 3 PRISM studies, 19% of study participants required a 60 mg dose to achieve adequate response to Palyngiq.

Palyngiq is indicated to reduce blood Phe concentrations in adults with phenylketonuria (PKU), who have uncontrolled blood Phe concentrations greater than 600 µmol/L on existing management. Palyngiq, a PEGylated recombinant phenylalanine ammonia lyase enzyme, is the first and only approved enzyme substitution therapy to target the underlying cause of PKU by helping the body to break down Phe.

- **BMN 307 gene therapy product candidate for phenylketonuria (PKU):** On September 24, 2020, the Company announced that it began dosing participants in PHEARLESS, the Phase 1/2 study of BMN 307. Both the FDA and EMA granted BMN 307 Orphan Drug Status. Additionally, the FDA has granted Fast Track status to BMN 307. Product for use in the Phase 1/2 study was made at commercial scale from BioMarin's award-winning gene therapy manufacturing facility.
- **BMN 331 gene therapy product candidate for Hereditary Angioedema (HAE):** IND-enabling studies are ongoing with BMN 331,

BioMarin's third gene therapy candidate, for the treatment of Hereditary Angioedema (HAE). BioMarin plans to leverage its broad expertise in developing gene therapies for severe hemophilia A and PKU to improve efficiencies in the development process of BMN 331.

- **DiNA-001 for MYBPC3 hypertrophic cardiomyopathy (HCM):** Pre-clinical studies are underway with DiNA-001 following a collaboration announced in May 2020 with DiNAQOR, a gene therapy platform company, to develop novel gene therapies to treat rare genetic cardiomyopathies. DiNAQOR received an undisclosed upfront payment and is eligible to receive development, regulatory and commercial milestones on product sales in addition to tiered royalties on worldwide sales.

BioMarin will host a conference call and webcast to discuss third quarter 2020 financial results today, Thursday, November 5, 2020 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: 3291898	Conference ID: 3291898

## 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, Research and Development Expense, Selling, General and Administrative Expense, Cost of Sales, GAAP Net Income, Non-GAAP Income, and other specified income statement guidance for the full-year 2020; the timing of BioMarin's clinical development and commercial prospects, including announcements of data from clinical studies and trials, including that the Company anticipates sharing top-line results from its Phase 3 study with valoctocogene roxaparvovec in early 2021; the clinical development and commercialization of BioMarin's product candidates and commercial products, including (i) BioMarin's plan to submit complete one-year Phase 3 data to the EMA in the second quarter of 2021, (ii) BioMarin's plan to resubmit its MAA for valoctocogene roxaparvovec to the EMA in the second quarter of 2021, and (iii) the potential approval and commercialization of BioMarin's product candidates, including vosoritide for the treatment of achondroplasia and valoctocogene roxaparvovec for the treatment of severe hemophilia A, including timing of such approval decisions.

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, as well as the potential impact of the COVID-19 pandemic on (i) BioMarin's ability to continue such preclinical studies and clinical trials and (ii) the timing of such 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, including the potential impact of the COVID-19 pandemic on the regulatory authorities' abilities to issue such decisions and the timing of such decisions; the market for each of these products; actual sales of BioMarin's commercial products and the impact that the COVID-19 pandemic may have on such sales; 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, 2020 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®, 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**  
**September 30, 2020 and December 31, 2019**  
(In thousands of U.S. dollars, except per share amounts)

	<b>September 30,</b>	<b>December 31,</b>
	<b>2020</b>	<b>2019(1)</b>
<b>ASSETS</b>	(unaudited)	
Current assets:		
Cash and cash equivalents	\$ 1,015,675	\$ 437,446
Short-term investments	489,998	316,361
Accounts receivable, net	411,712	377,404
Inventory	700,847	680,275
Other current assets	120,747	130,657
Total current assets	2,738,979	1,942,143
Noncurrent assets:		
Long-term investments	265,122	411,978
Property, plant and equipment, net	1,015,062	1,010,868
Intangible assets, net	427,172	456,580
Goodwill	196,199	197,039
Deferred tax assets	1,396,547	549,422
Other assets	119,009	122,009
Total assets	\$ 6,158,090	\$ 4,690,039
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 480,403	\$ 570,621
Short-term convertible debt, net	374,290	361,882
Total current liabilities	854,693	932,503
Noncurrent liabilities:		
Long-term convertible debt, net	1,074,164	486,238
Long-term contingent consideration	54,103	50,793
Other long-term liabilities	121,237	98,124
Total liabilities	\$ 2,104,197	\$ 1,567,658
Stockholders' equity:		
Common stock, \$0.001 par value: 500,000,000 shares authorized; 181,492,344 and 179,838,114 shares issued and outstanding, respectively.	181	180
Additional paid-in capital	4,937,791	4,832,707
Company common stock held by Nonqualified Deferred Compensation Plan	(10,756)	(9,961)
Accumulated other comprehensive income	10,385	20,164
Accumulated deficit	(883,708)	(1,720,709)
Total stockholders' equity	4,053,893	3,122,381
Total liabilities and stockholders' equity	\$ 6,158,090	\$ 4,690,039

(1) December 31, 2019 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, 2019, filed with the U.S. Securities and Exchange Commission (SEC) on February 27, 2020.

**BIOMARIN PHARMACEUTICAL INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**

**Nine Months Ended September 30, 2020 and 2019**

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
	(unaudited)	(unaudited)	(unaudited)	(unaudited)
<b>REVENUES:</b>				
Net product revenues	\$ 460,741	\$ 450,900	\$ 1,368,816	\$ 1,224,458
Royalty and other revenues	16,043	10,197	39,522	25,147
Total net revenues	476,784	461,097	1,408,338	1,249,605
<b>OPERATING EXPENSES:</b>				
Cost of sales	188,793	96,949	398,134	263,567
Research and development	147,053	172,963	471,449	542,195
Selling, general and administrative	179,450	170,112	542,157	493,024
Intangible asset amortization and contingent consideration	17,429	17,063	48,018	57,114
Gain on sale of nonfinancial assets	—	—	(59,495)	(15,000)
Total operating expenses	532,725	457,087	1,400,263	1,340,900
<b>INCOME (LOSS) FROM OPERATIONS</b>	(55,941)	4,010	8,075	(91,295)
Equity in the loss of BioMarin/Genzyme LLC	(921)	(551)	(1,077)	(780)
Interest income	4,004	5,340	13,539	17,537
Interest expense	(9,597)	(2,937)	(24,560)	(16,530)
Other income, net	1,239	3,960	1,886	6,038
<b>INCOME (LOSS) BEFORE INCOME TAXES</b>	(61,216)	9,822	(2,137)	(85,030)
Benefit from income taxes	(846,019)	(45,214)	(839,138)	(46,158)
<b>NET INCOME (LOSS)</b>	784,803	55,036	837,001	(38,872)
<b>NET INCOME (LOSS) PER SHARE, BASIC</b>	\$ 4.33	\$ 0.31	\$ 4.63	\$ (0.22)
<b>NET INCOME (LOSS) PER SHARE, DILUTED</b>	\$ 4.01	\$ 0.30	\$ 4.39	\$ (0.22)
Weighted average common shares outstanding, basic	181,142	179,289	180,592	178,873
Weighted average common shares outstanding, diluted	197,674	185,924	194,959	178,873

The following table presents Net Product Revenues by Product:

**Net Product Revenues by Product**

(In millions of U.S. dollars)

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2020	2019	% Change	2020	2019	% Change
	(unaudited)	(unaudited)		(unaudited)	(unaudited)	
PKU franchise	\$ 170.2	\$ 144.7	18 %	\$ 490.1	\$ 396.0	24 %
Vimizim	147.9	163.5	(10) %	401.8	412.0	(2) %
Naglazyme	76.3	94.4	(19) %	271.6	279.5	(3) %
Brineura	25.4	19.8	28 %	75.2	46.8	61 %
Firdapse <sup>(1)</sup>	—	5.7	(100) %	1.2	16.3	(93) %
Net Product Revenues Marketed by BioMarin	419.8	428.1		1,239.9	1,150.6	
Aldurazyme Net Product Revenues						

Marketed by Genzyme	40.9	22.8	79 %	128.9	73.9	74 %
Total Net Product Revenues	<u>\$ 460.7</u>	<u>\$ 450.9</u>		<u>\$ 1,368.8</u>	<u>\$ 1,224.5</u>	

(1) In January 2020, BioMarin divested the Firdapse assets to a third party in a sale transaction. The sale is reflected in the Company's consolidated financial statements for the three and nine months ending September 30, 2020; and as a result of the transaction BioMarin will not recognize Net Product Revenues from Firdapse in the future.

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)**

	<b>Three Months Ended September 30,</b>			<b>Nine Months Ended September 30,</b>		
	<b>2020</b>	<b>2019</b>	<b>% Change</b>	<b>2020</b>	<b>2019</b>	<b>% Change</b>
	(unaudited)	(unaudited)		(unaudited)	(unaudited)	
Kuvan	\$ 124.1	120.6	3 %	\$ 368.7	340.8	8 %
Palynziq	46.1	24.1	91 %	121.4	55.2	120 %
Total PKU franchise	<u>\$ 170.2</u>	<u>\$ 144.7</u>	18 %	<u>\$ 490.1</u>	<u>\$ 396.0</u>	24 %

**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)**

<b>Three Months Ended September 30,</b>		<b>Nine Months Ended September 30,</b>		<b>Guidance Year Ending</b>
<b>2020</b>	<b>2019</b>	<b>2020</b>	<b>2019</b>	<b>December 31, 2020</b>

<b>GAAP Net Income (Loss)</b>	<b>\$ 784.8</b>	<b>\$ 55.0</b>	<b>\$ 837.0</b>	<b>\$ (38.9)</b>	<b>\$ 760.0</b>	<b>—</b>	<b>\$ 820.0</b>
Interest expense, net	5.6	(2.4)	11.0	(1.0)	13.0	—	11.0
Benefit from income taxes	(846.0)	(45.2)	(839.1)	(46.2)	(845.8)	—	(838.8)
Depreciation expense	11.1	14.4	31.1	42.3	50.0	—	47.0
Amortization expense	15.5	16.3	46.7	37.2	63.0	—	61.0
Stock-based compensation expense	50.2	39.2	142.2	121.8	195.4	—	185.4
Contingent consideration expense	1.9	0.8	1.3	19.9	2.0	—	2.0
Provision for inventory reserve, net (1)	75.6	—	75.6	—	75.6	—	75.6
Gain on sale of nonfinancial assets	—	—	(59.5)	(15.0)	(59.5)	—	(59.5)
Licensed In-Process R&D (2)	—	—	26.3	—	26.3	—	26.3
Non-GAAP Income	<u>\$ 98.7</u>	<u>\$ 78.1</u>	<u>\$ 272.6</u>	<u>\$ 120.1</u>	<u>\$ 280.0</u>	<u>—</u>	<u>\$ 330.0</u>

(1) Represents a \$81.2 million charge related to pre-launch valoctocogene roxaparvec inventory, net of stock-based compensation, as a result of the unexpected delays in anticipated regulatory approvals.

(2) Represents the upfront license fee paid to a third party and recognized as R&D expense in the second quarter of 2020.

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,

	2020				2019			
	GAAP Reported	Adjustments		Non-GAAP	GAAP Reported	Adjustments		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	\$ 188.8	\$ —	\$ (86.0)	\$ 102.8	\$ 96.9	\$ —	\$ (4.1)	\$ 9
Research and development	147.1	(5.8)	(15.7)	125.6	173.0	(6.4)	(14.3)	15
Selling, general and administrative	179.5	(5.3)	(24.1)	150.1	170.1	(8.0)	(20.8)	14
Intangible asset amortization and contingent consideration	17.4	(15.5)	(1.9)	—	17.1	(16.3)	(0.8)	—
Gain on sale of nonfinancial assets	—	—	—	—	—	—	—	—
Interest expense, net	(5.6)	5.6	—	—	2.4	(2.4)	—	—
Benefit from income taxes	(846.0)	846.0	—	—	(45.2)	45.2	—	—

GAAP Net									
Income /Non-									
GAAP Income	\$ 784.8	\$ (813.8)	\$ 127.7	\$ 98.7	\$ 55.0	\$ (16.9)	\$ 40.0	\$ 7	

**Nine months ended September 30,**

	2020				2019			
	Adjustments			Non-GAAP	Adjustments			Non-GA
	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	\$ 398.1	\$ 0.0	\$ (96.0)	\$ 302.1	\$ 263.6	\$ 0.0	(12.6)	\$ 25
Research and development	471.4	(15.2)	(71.7)	384.5	542.2	(23.1)	(43.1)	47
Selling, general and administrative	542.2	(15.9)	(76.4)	449.9	493.0	(19.2)	(66.1)	40
Intangible asset amortization and contingent consideration	48.0	(46.7)	(1.3)	—	57.1	(37.2)	(19.9)	
Gain on sale of nonfinancial assets	(59.5)	—	59.5	—	(15.0)	—	15.0	
Interest expense, net	(11.0)	11.0	—	—	1.0	(1.0)	—	
Benefit from income taxes	(839.1)	839.1	—	—	(46.2)	46.2	—	
GAAP Net Income (Loss)/Non-GAAP Income	837.0	(750.3)	185.9	272.6	(38.9)	32.3	126.7	12

SOURCE BioMarin Pharmaceutical Inc.

<https://investors.biopharm.com/2020-11-05-BioMarin-Announces-Third-Quarter-2020-Total-Revenues-of-477-Million>