BioMarin Announces Fourth Quarter and Record Full-year 2019 Financial Results

- Full-year 2019 Total Revenues Increased 14% to $1.7 billion
- 17% Increase in Net Product Revenues from Products Marketed by BioMarin to $1.6 billion in Full-year 2019
- 2020 Full-year Total Revenue Growth Expected to be Between 14% and 20%
- For the Full-year 2020 BioMarin Expects for the First Time to be Profitable on a GAAP Basis
- U.S. and European Marketing Submissions for Valoctocogene Roxaparvovec for Severe Hemophilia A Accepted by FDA and EMA under Priority Review and Accelerated Assessment, Respectively; Approval Decisions Expected in Second Half of 2020
- Phase 3 Study with Vosoritide for Achondroplasia Demonstrated Highly Statistically Significant Increase (p<0.0001) in Growth Velocity in Children Treated Over One Year
- Phase 1/2 Study with BMN 307 Gene Therapy Product Candidate for Phenylketonuria Screening Participants

SAN RAFAEL, Calif., Feb. 26, 2020 /PRNewswire/ --

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

	Three Months Ended December 31,			Twelve Months Ended December 31,		
	2019	2018	% Change	2019	2018	% Change
Total Revenues	$ 454.4	$ 353.2	29 %	$ 1,704.0	$ 1,491.2	14 %
Net Product Revenues Marketed by BioMarin (1)	412.7	329.8	25 %	1,563.2	1,335.3	17 %
Vimizim Net Product Revenues	132.3	114.0	16 %	544.3	482.0	13 %
Kuvan Net Product Revenues	122.6	112.2	9 %	463.4	433.6	7 %
Naglazyme Net Product Revenues	94.8	76.7	24 %	374.3	345.9	8 %
Palynziq Net Product Revenues	31.7	8.1	N/M	86.9	12.2	N/M
Brineura Net Product Revenues	25.2	12.2	107 %	72.0	39.9	80 %
Aldurazyme Net Product Revenues	23.9	17.4	37 %	97.8	135.1	(28) %
GAAP Net Income (Loss)	$ 15.0	$ (3.7)		$ (23.8)	$ (77.2)	
GAAP Net Income (Loss) per Share – Basic	$ 0.08	$ (0.02)		$ (0.13)	$ (0.44)	
GAAP Net Income (Loss) per Share – Diluted	$ 0.08	$ (0.03)		$ (0.13)	$ (0.44)	
Non-GAAP Income (Loss) (2)	$ 46.4	$ (10.9)		$ 166.6	$ 90.9	

	December 31, 2019	December 31, 2018
Cash, cash equivalents and investments	$ 1,165.8	$ 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 fourth quarter and full year 2019.

Total Net Product Revenues for the fourth quarter of 2019 increased to $436.6 million, compared to $347.2 million for the fourth quarter of 2018. The increase in Net Product Revenues was attributed to the following:

- Palynziq Net Product Revenues increased by $23.6 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 approval in May 2019 from the European Commission (EC) and commercial sales in Europe commenced in the fourth quarter of 2019;
- Vimizim Net Product Revenues increased by $18.3 million, or 16%, driven primarily by patient growth in the U.S. and orders from Brazil;
- Naglazyme Net Product Revenues increased by $18.1 million, or 24%, primarily due to orders from Brazil;
- Brineura Net Product Revenues increased by $13.0 million, or 107%, due in large part by patient growth across all regions particularly Europe;
- Kuvan Net Product Revenues increased by $10.4 million, or 9%, primarily driven by an increase in patients in North America; and
- Aldurazyme Net Product Revenues increased $6.5 million, or 37%, due to higher sales volume to Sanofi Genzyme.

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

- increased gross profits of $80.3 million primarily driven by increased product sales; partially offset by
- higher selling, general and administrative (SG&A) expense related to pre-commercialization activities for valoctocogene roxaparvovec, and support of the EU commercial launch and continued U.S. expansion of Palynziq.

Non-GAAP Income for the fourth quarter of 2019 increased to $46.4 million, compared to Non-GAAP Loss of $10.9 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 decreased research and development expense, partially offset by higher SG&A expense.

As of December 31, 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 2019 results, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "Our performance in 2019 reflects the clinical, regulatory and financial goals we set for ourselves a year ago. We completed enrollment of the Phase 3 Interim Analysis cohort with valoctocogene roxaparvovec and met the criteria for expedited review of marketing applications in the United States and Europe. With those applications now under review by global health authorities, we await the potential approval of valoctocogene roxaparvovec, the first gene therapy product to be reviewed for the treatment of severe hemophilia A. Another significant success in 2019 was the completion of and results from our global Phase 3 study with vosoritide for achondroplasia. With a high degree of statistical significance ($p<0.0001$) the primary endpoint of increase in growth velocity from baseline over a one year period was demonstrated with vosoritide. Based on these results, the Company plans to meet with health authorities in the first half of 2020 to discuss plans for submitting marketing applications later this year. Another study with vosoritide, the global Phase 2 in infants and young children (less than 60 months old), is continuing enrollment. All subjects from ages 6 months through 5 years are almost completely enrolled. The youngest cohort, infants up to 6 months old, is actively enrolling. Interest in this study with very young children has been extremely robust, demonstrating that families are keen to seek treatment for their children as early as possible."

Mr. Bienaimé continued, "In addition to these later-stage regulatory and clinical milestones in 2019, we made significant progress advancing our early-stage pipeline. Building on the success of our phenylketonuria (PKU) franchise with Palynziq and Kuvan, we announced in January that both the United States and the United Kingdom health authorities had given the go-ahead to start dosing patients with PKU with our BMN 307 gene therapy in a Phase 1/2 study. We plan to treat patients with BMN 307 in the first quarter using product made at commercial scale from our award-winning gene therapy manufacturing facility. Other additions to the pipeline announced at R&D Day last November included, BMN 331 gene therapy for hereditary angioedema and vosoritide for dominantly inherited short stature. Finally, our most recently approved product, Palynziq for the treatment of PKU, continues along a very strong growth trajectory since approval in 2018 in the U.S. We have been very pleased with the pace of the U.S. launch, as we ended 2019 with 762 patients on reimbursed Palynziq, and an additional 143 naïve patients having completed enrollment and awaiting their first injection. Based on the tremendous interest in treatment with Palynziq, we provided full-year guidance for Palynziq Net Product Revenues of between $180 million to $210 million for full-year 2020, representing significant growth over 2019. Our revenue growth and improvement in profitability also increased our operating cash flows, as shown by the growth of our total cash and investments for the second straight quarter. Having achieved all of our key clinical, regulatory and financial goals in 2019, 2020 looks poised to be one of our most significant value-creating years to date."

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

Item	2020 Guidance				
Total Revenues	$1,950		to	$2,050	
Vimizim Net Product Revenues	$560		to	$610	
Kuvan Net Product Revenues	$430		to	$480	
Naglazyme Net Product Revenues	$380		to	$420	
Palynziq Net Product Revenues	$180		to	$210	
Brineura Net Product Revenues	$85		to	$115	
Cost of Sales (% of Total Revenues)	20	%	to	21	%
Research and Development Expense	$675		to	$725	
Selling, General and Administrative Expense	$780		to	$830	
GAAP Net Income (1)	$20		to	$80	
Non-GAAP Income (2)	$260		to	$310	

(1) 2020 GAAP Net Income guidance does not reflect the potential impact on non-cash GAAP income tax associated with the tax effects of potential intra-entity intangible asset transfers between BioMarin entities as a result of changing international tax laws. Any such changes, if implemented, are not expected to have an impact on operations or cash flows in 2020 but may have an impact on GAAP Net Income in the form of an income tax benefit of potentially greater than $500 million.

(2) 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 severe hemophilia A:** On February 20, 2020, FDA accepted for Priority Review the Biologics License Application (BLA) for valoctocogene roxaparvovec. The Prescription Drug User Fee Act (PDUFA), or target action date for the BLA, has been set for August 21, 2020. On December 23, 2019, the Company announced that the European Medicines Agency (EMA) validated the Company's Marketing Authorization Application (MAA) for valoctocogene roxaparvovec which has been in review under accelerated assessment since January. The submissions are based on the Phase 3 interim analysis and the updated three-year Phase 1/2 data of patients treated with valoctocogene roxaparvovec. Both submissions represent the first time a gene therapy product for any type of hemophilia indication is under review for marketing authorization by health authorities.

  BioMarin has dosed 134 study participants in the full GENEr8-1 Phase 3 study with 52-week results expected in the first quarter of 2021. Although the trial is open label, BioMarin has implemented a data access plan designed to substantially 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.

  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 phenylalanine (Phe) concentrations in adult patients with PKU, was added to BioMarin's commercial product portfolio upon its U.S. approval May 2018. As of December 31, 2019, 762 patients were on reimbursed Palynziq, with an additional 143 naïve patients enrolled and awaiting their first treatment with commercial Palynziq. Of the 762 patients on therapy at the end of the fourth quarter, 625 were formerly naïve patients and 137 had transitioned from clinical studies. 143 enrolled naive patients were awaiting their first administration of Palynziq as of the end of the fourth quarter. Of the 125 PKU clinics in the U.S., 97 sites had active patients currently on therapy as of December 31, 2019.

  On May 6, 2019, the 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:** On December 16, 2019, the Company reported positive final results from its randomized, double-blind, placebo-controlled Phase 3 study evaluating the efficacy and safety of vosoritide. The placebo-adjusted increased change from baseline in growth velocity after one year of treatment with vosoritide, the primary endpoint, was 1.6 cm/yr ($p<0.0001$). Vosoritide is an investigational, once daily injection analog of C-type Natriuretic Peptide (CNP). The study enrolled 121 children aged 5 to 14 with achondroplasia, the most common form of disproportionate short stature. The results were consistent across the broad patient population studied. Vosoritide was generally well tolerated with no clinically significant blood pressure decreases. Based on these results, the Company plans to meet with health authorities in the first half of 2020 to discuss plans for submitting marketing applications later this year.

On November 14, 2019, the Company provided an update on the ongoing Phase 2 study of vosoritide which demonstrated over 54 months that children in cohort 3 (N=10) of the study, at a dose of 15 µg/kg/day, achieved a statistically significant (p< 0.005) cumulative mean additional height gain of 9.0 cm compared to children, matched for age and gender, in a new comprehensive natural history achondroplasia dataset (N=619). 2.2 cm of this additional increase occurred in the last 12 months further informing our understanding of vosoritide's ongoing treatment impact. These data are expected to corroborate maintenance of effect at the time of anticipated marketing application submissions later this year.

The vosoritide development program includes four distinct areas of focus to support global approval. In addition to the completed Phase 3 study and ongoing Phase 2 study in children ages 5-14 years, the global program includes a large contemporaneous natural history study which is underway.

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. Cohorts 1 and 2 include children from ages 6 months through 5 years of age, is near enrollment completion. Cohort 3, includes children ages 0 to 6 months old and, is actively enrolling.

- **BMN 307 gene therapy product candidate for phenylketonuria (PKU):** On January 13, 2020 the Company announced that both the FDA and the Medicines and Healthcare Products Regulatory Agency (MHRA) in the U.K. have granted the Company Investigational New Drug (IND) status and approved its Clinical Trial Application (CTA), respectively, for BMN 307.

  The Company expects to start dosing patients in PHEARLESS, a Phase 1/2 study, in the first quarter of 2020 with product made at commercial scale from its award-winning gene therapy manufacturing facility. Both the FDA and EMA have granted BMN 307 Orphan Status.

  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.

- **BMN 331 gene therapy product candidate for Hereditary Angioedema (HAE):** On November 14, 2019, the Company introduced its third gene therapy candidate, BMN 331, for the treatment of Hereditary Angioedema (HAE). BioMarin plans to build on its ever wider and deeper experience in developing gene therapies for severe hemophilia A and PKU to improve efficiencies in the development process, and optimize capsid and transgene design. The Company expects to begin IND-enabling studies in early to mid-2020.

- **Vosoritide for the treatment of Dominantly Inherited Short Stature (DISS):** On November 14, 2019, the Company announced that vosoritide will be studied in broader genetic statural abnormalities starting with dominantly inherited short stature (DISS), as part of a research collaboration with Children's National Hospital. The Company plans to build on its learnings with vosoritide in achondroplasia and look for efficiencies in the development process, particularly around pre-clinical research and manufacturing. The Company expects the trial with vosoritide for DISS to begin mid-2020.

- **Gene Therapy manufacturing productivity increases capacity:** On January 13, 2020, the Company announced that significant improvements in productivity in the gene therapy facility had increased capacity for up to 10,000 patients per year, depending on dose and product mix.

BioMarin will host a conference call and webcast to discuss fourth quarter and full year 2019 financial results and 2020 guidance today, Wednesday, February 26, 2020 at 4:30 p.m. ET. This event can be accessed on the investor section of the BioMarin website at www.biomarin.com.

U.S./Canada Dial-in Number: 866.502.9859	Replay Dial-in Number: 855.859.2056
International Dial-in Number: 574.990.1362	Replay International Dial-in Number: 404.537.3406
Conference ID: 9967337	Conference ID: 9967337

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.biomarin.com.

Forward-Looking Statements

This press release and the associated conference call and webcast contain forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about: the expectations of Total Revenues, Net Product Revenues, Research and Development Expense, Selling, General and Administrative Expense, Cost of Sales, GAAP Net Income, Non-GAAP Income, other specified income statement guidance for the full-year 2020, and 2020 being poised to be one of the Company's most significant value-creating years to date; the financial performance of BioMarin as a whole, including BioMarin's expectation that it will become profitable on a GAAP basis for the first time in 2020; BioMarin's potential for significant growth and expansion; BioMarin anticipating significant milestones over the coming months; the timing of BioMarin's clinical development and commercial prospects, including (i) the Company's expectation that it will start dosing patients in the PHEARLESS study in the first quarter of 2020, the Company's expectation that it will begin IND-enabling studies for BMN 331 for HAE in mid-2020, and the Company's expectation that vosoritide will be studied in broader genetic statural deficiencies with DISS as part of a research collaboration with Children's National Hospital, with a clinical trial expected to begin mid-2020, (ii) BioMarin's clinical studies and trials, (iii) completion of enrollment of those studies and trials, and (iv) announcements of data from those studies and trials, including BioMarin's Phase 2 program with valoctocogene roxaparvovec; the clinical development and commercialization of BioMarin's product candidates and commercial products, including (i) BioMarin's plans to meet with health authorities in the first half of 2020 to discuss plans for submitting marketing applications for vosoritide for children with achondroplasia, (ii) the productivity and capacity of its gene therapy manufacturing facility, (iii) the potential approval and commercialization of BioMarin's product candidates, including valoctocogene roxaparvovec for the treatment of severe hemophilia A, and (iv) the Company's securing of reimbursement for Palynziq on a country-by-country basis in the EU and Palynziq's revenue from the EU in 2020 and the anticipation of meaningful revenue contributions from this region 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 September 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 PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
December 31, 2019 and 2018
(In thousands of U.S. dollars, except per share amounts)

	December 31, 2019 (1)	December 31, 2018 (2)
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 437,446	$ 493,982
Short-term investments	316,361	590,326
Accounts receivable, net	377,404	342,633
Inventory	680,275	530,871
Other current assets	130,657	98,403
Total current assets	1,942,143	2,056,215
Noncurrent assets:		
Long-term investments	411,978	235,864
Property, plant and equipment, net	1,010,868	948,682
Intangible assets, net	456,580	491,808
Goodwill	197,039	197,039
Deferred tax assets	549,422	460,952
Other assets	122,009	36,568
Total assets	$ 4,690,039	$ 4,427,128
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 570,621	$ 437,290
Short-term convertible debt, net	361,882	—
Short-term contingent consideration	—	85,951
Total current liabilities	932,503	523,241
Noncurrent liabilities:		
Long-term convertible debt, net	486,238	830,417
Long-term contingent consideration	50,793	46,883
Other long-term liabilities	98,124	58,647
Total liabilities	$ 1,567,658	$ 1,459,188
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 179,838,114 and 178,252,954 shares issued and outstanding, respectively.	180	178
Additional paid-in capital	4,832,707	4,669,926
Company common stock held by Nonqualified Deferred Compensation Plan	(9,961)	(13,301)
Accumulated other comprehensive income	20,164	5,271
Accumulated deficit	(1,720,709)	(1,694,134)
Total stockholders' equity	3,122,381	2,967,940
Total liabilities and stockholders' equity	$ 4,690,039	$ 4,427,128

(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, 2019.

BIOMARIN PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
Three and Twelve Months Ended December 31, 2019 and 2018
(In thousands of U.S. dollars, except per share amounts)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	2018	2019	2018 (1)
	(unaudited)	(unaudited)	(unaudited)	
REVENUES:				
Net product revenues	$ 436,585	$ 347,151	$ 1,661,043	$ 1,470,356
Royalty and other revenues	17,858	6,063	43,005	20,856
Total net revenues	454,443	353,214	1,704,048	1,491,212
OPERATING EXPENSES:				
Cost of sales	95,899	75,019	359,466	315,264
Research and development	172,812	175,390	715,007	696,328
Selling, general and administrative	187,900	164,171	680,924	604,353
Intangible asset amortization and contingent consideration	16,994	6,782	74,108	48,791
Gain on sale of intangible assets	(10,000)	(30,000)	(25,000)	(50,000)
Total operating expenses	463,605	391,362	1,804,505	1,614,736
LOSS FROM OPERATIONS	(9,162)	(38,148)	(100,457)	(123,524)
Equity in the loss of BioMarin/Genzyme LLC	193	(46)	(587)	(553)
Interest income	5,211	5,690	22,748	22,831
Interest expense	(6,930)	(7,746)	(23,460)	(43,664)
Other income, net	907	(3,061)	6,945	2,205
INCOME (LOSS) BEFORE INCOME TAXES	(9,781)	(43,311)	(94,811)	(142,705)
Benefit from income taxes	(24,805)	(39,661)	(70,963)	(65,494)
NET INCOME (LOSS)	15,024	(3,650)	(23,848)	(77,211)
NET INCOME (LOSS) PER SHARE, BASIC	$ 0.08	$ (0.02)	$ (0.13)	$ (0.44)
NET INCOME (LOSS) PER SHARE, DILUTED	$ 0.08	$ (0.03)	$ (0.13)	$ (0.44)
Weighted average common shares outstanding, basic	179,531	177,963	179,039	177,061
Weighted average common shares outstanding, diluted	182,412	178,143	179,039	177,268

(1) December 31, 2018 totals 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 SEC on February 28, 2019.

The following table presents Net Product Revenues by Product:

Net Product Revenues by Product

(In millions of U.S. dollars)

	Three Months Ended December 31,			Twelve Months Ended December 31,		
	2019	2018	% Change	2019	2018 (1)	% Change
	(unaudited)	(unaudited)		(unaudited)		
Brineura	$ 25.2	$ 12.2	107 %	$ 72.0	$ 39.9	80 %
Firdapse (2)	6.1	6.6	(8) %	22.3	21.7	3 %
Naglazyme	94.8	76.7	24 %	374.3	345.9	8 %
PKU franchise	154.3	120.3	28 %	550.3	445.8	23 %
Vimizim	132.3	114.0	16 %	544.3	482.0	13 %
Net Product Revenues Marketed by BioMarin	412.7	329.8		1,563.2	1,335.3	
Aldurazyme Net Product Revenues Marketed by Genzyme	23.9	17.4	37 %	97.8	135.1	(28) %
Total Net Product Revenues	$ 436.6	$ 347.2		$ 1,661.0	$ 1,470.4	

(1) December 31, 2018 totals 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 SEC on February 28, 2019.

(2) In January 2020, BioMarin divested the Firdapse assets to a third party in a sale transaction. The sale will be reflected in the Company's consolidated financial statements for the three months ending March 31, 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)

	Three Months Ended December 31,			Twelve Months Ended December 31,		
	2019	2018	% Change	2019	2018 (1)	% Change
	(unaudited)	(unaudited)		(unaudited)		
Kuvan	$ 122.6	112.2	9 %	$ 463.4	433.6	7 %
Palynziq	31.7	8.1	NM	86.9	12.2	NM
Total PKU franchise	$ 154.3	$ 120.3	28 %	$ 550.3	$ 445.8	23 %

(1) December 31, 2018 totals 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 SEC on February 28, 2019.

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)

	Three Months Ended December 31,		Twelve Months Ended December 31,		Guidance Year Ending		
	2019	2018	2019	2018	December 31, 2020		
GAAP Net Income (Loss)	**$ 15.0**	**$ (3.7)**	**$ (23.8)**	**$ (77.2)**	**$ 20.0**	**$ —**	**$ 80.0**
Interest expense, net	1.7	2.1	0.7	20.8	3.0	—	2.0
Benefit from income taxes	(24.8)	(39.7)	(71.0)	(65.5)	5.0	—	11.0
Depreciation expense	9.5	17.1	51.8	65.2	50.0	—	47.0
Amortization expense	16.3	7.6	53.5	30.3	63.0	—	61.0
Stock-based compensation expense	38.0	36.5	159.8	148.8	177.0	—	167.0
Contingent consideration expense	0.7	(0.8)	20.6	18.5	2.0	—	2.0
Gain on sale of intangible assets	(10.0)	(30.0)	(25.0)	(50.0)	(60.0)	—	(60.0)
Non-GAAP Income (Loss)	$ 46.4	$ (10.9)	$ 166.6	$ 90.9	$ 260.0	—	$ 310.0

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 December 31,

	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	**$ 95.9**	$ —	$ (3.5)	$ 92.4	**$ 75.0**	$ —	$ (3.1)	$ 71.9
Research and development	**172.8**	(3.8)	(13.5)	155.5	**175.4**	(11.2)	(14.5)	149.7
Selling, general and administrative	**187.9**	(5.7)	(21.0)	161.2	**164.2**	(5.9)	(18.9)	139.4
Intangible asset amortization and contingent consideration	**17.0**	(16.3)	(0.7)	—	**6.8**	(7.6)	0.8	—
Gain on sale of intangible assets	**(10.0)**	—	10.0	—	**(30.0)**	—	30.0	—
Interest expense, net	**(1.7)**	1.7	—	—	**(2.1)**	2.1	—	—
Benefit from income taxes	**(24.8)**	24.8	—	—	**(39.7)**	39.7	—	—
GAAP Net Income (Loss)/Non-GAAP Income (Loss)	**$ 15.0**	$ 2.7	$ 28.7	$ 46.4	**$ (3.7)**	$ (12.9)	$ 5.7	$ (10.9)

Twelve months ended December 31,

	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	**$ 359.5**	$ —	$ (16.1)	$ 343.4	**$ 315.3**	$ —	$ (13.5)	$ 301.8
Research and development	**715.0**	(26.9)	(56.6)	631.5	**696.3**	(36.6)	(57.6)	602.1
Selling, general and administrative	**680.9**	(24.9)	(87.1)	568.9	**604.4**	(28.6)	(77.7)	498.1
Intangible asset amortization and contingent consideration	**74.1**	(53.5)	(20.6)	—	**48.8**	(30.3)	(18.5)	—
Gain on sale of intangible assets	**(25.0)**	—	25.0	—	**(50.0)**	—	50.0	—
Interest expense, net	**(0.7)**	0.7	—	—	**(20.8)**	20.8	—	—
Benefit from income taxes	**(71.0)**	71.0	—	—	**(65.5)**	65.5	—	—
GAAP Net Loss/Non-GAAP Income	**$ (23.8)**	$ 35.0	$ 155.4	$ 166.6	**$ (77.2)**	$ 50.8	$ 117.3	$ 90.9

Contact:
Investors:
Traci McCarty
BioMarin Pharmaceutical Inc.
(415) 455-7558

Media:
Debra Charlesworth
BioMarin Pharmaceutical Inc.
(415) 455-7451

SOURCE BioMarin Pharmaceutical Inc.