BioMarin Announces Fourth Quarter and Record Full-year 2020 Financial Results and Corporate Updates

- Full-year 2020 Total Revenues Increased 9% to $1.86 billion

- Company Dose-escalates in PHEarless, a Phase 1/2 Study with BMN 307 Gene Therapy for the Treatment of Phenylketonuria (PKU); the Third Potential Therapeutic Modality in its PKU Franchise

- During the Fourth Quarter, BioMarin Announced Benefit Maintained for Over Two Years in Children with Achondroplasia Treated with Vosoritide in Phase 3 Extension Study

- 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 June 2021

- In January 2021, BioMarin Announced Positive Phase 3 Results with Valoctocogene Roxaparvovec in Adults with Severe Hemophilia A; Study Met All Primary and Secondary Efficacy Endpoints in One-Year Data Set; Mean Annualized Bleed Rate was 0.9 in Subset Dosed More than Two Years Ago

- 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) Targeted in 2Q21

SAN RAFAEL, Calif., Feb. 25, 2021 /PRNewswire/ --

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

	Three Months Ended December 31,			Twelve Months Ended December 31,		
	2020	2019	% Change	2020	2019	% Change
Total Revenues	$ 452.1	$ 454.4	(1) %	$ 1,860.5	$ 1,704.0	9 %
Net Product Revenues Marketed by BioMarin [1]	435.8	412.7	6 %	1,675.8	1,563.2	7 %
Vimizim Net Product Revenues	142.5	132.3	8 %	544.4	544.3	— %
Kuvan Net Product Revenues	89.0	122.6	(27) %	457.7	463.4	(1) %
Naglazyme Net Product Revenues	119.7	94.8	26 %	391.3	374.3	5 %
Palynziq Net Product Revenues	49.6	31.7	56 %	171.0	86.9	97 %
Brineura Net Product Revenues	35.0	25.2	39 %	110.2	72.0	53 %
Aldurazyme Net Product Revenues	1.2	23.9	(95) %	130.1	97.8	33 %
GAAP Net Income (Loss)	$ 22.1	$ 15.0		$ 859.1	$ (23.8)	
GAAP Net Income (Loss) per Share – Basic	$ 0.12	$ 0.08		$ 4.75	$ (0.13)	
GAAP Net Income (Loss) per Share – Diluted	$ 0.12	$ 0.08		$ 4.53	$ (0.13)	
Non-GAAP Income [2]	$ 39.5	$ 46.4		$ 312.2	$ 166.6	

	December 31, 2020	December 31, 2019
Cash, cash equivalents and investments	$ 1,350.9	$ 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. 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 twelve months ending December 31, 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/Loss, excluding net interest expense, provision for (benefit from) income taxes, depreciation expense, amortization expense, stock-based compensation expense, contingent consideration expense and, in certain periods, certain other specified items. Refer to Non-GAAP Information beginning on page 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 ended December 31, 2020.

Net Product Revenues for the fourth quarter of 2020 were essentially flat as compared to the fourth quarter of 2019. The change in Net Product Revenues was primarily attributed to the following:

- Kuvan Net Product Revenues decreased by $33.6 million, primarily due to the U.S. loss of market exclusivity in October 2020 resulting from generic competition; and
- Aldurazyme Net Product Revenues decreased by $22.7 million due to timing of product fulfillment to Genzyme. Aldurazyme is marketed by Genzyme and BioMarin Aldurazyme revenues are driven by the timing of when the product is released and control is transferred to Genzyme. Revenues for the fourth quarter of 2020 were comparatively lower than 2019 due to such timing. Based on data provided to us by Genzyme, patients receiving commercial Aldurazyme increased by 10% during 2020; partially offset by
- Naglazyme and Vimizim Net Product Revenues increased by an aggregate of $35.1 million primarily due to timing of sales in the Middle East and Latin America;
- Palynziq Net Product Revenues increased by $17.9 million driven by a combination of revenue from U.S. patients achieving maintenance dosing and new patients initiating therapy; and
- Brineura Net Product Revenues increased by $9.8 million driven by growth in the number of patients in all regions.

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

- decreased research and development (R&D) expense of $16.1 million primarily due to lower clinical activity spend for valoctocogene roxaparvovec gene therapy programs and decreased tralesinidase alfa costs as the program was licensed to a third-party in 2019; and
- an increase in the benefit from income taxes of $37.5 million primarily due to the change in the jurisdictional mix of earnings and the related tax impact from the completion of an intra-entity transfer of certain intellectual property rights to

an Irish subsidiary where the Company's ex-US regional headquarters are located during the third quarter of 2020; partially offset by

- an increase in Cost of Sales of $30.2 million primarily due to inventory reserves and higher sales volumes of products with lower margins.

Non-GAAP Income for the fourth quarter of 2020 decreased to $39.5 million, compared to Non-GAAP Income of $46.4 million for the same period in 2019. The decrease in Non-GAAP Income for the quarter, compared to the same period in 2019, was primarily attributed to lower gross profits and higher SG&A expenses, partially offset by lower R&D expenses.

As of December 31, 2020, BioMarin had cash, cash equivalents and investments totaling $1.35 billion, which includes net proceeds of $535.8 million from the Company's May 2020 convertible debt offering, as compared to $1.17 billion as of 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 full-year 2020 results, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "Despite the impact in 2020 from the COVID-19 pandemic and a delay in the potential approval of valoctocogene roxaparvovec for severe hemophilia A, demand for our current product portfolio continued to drive steady revenue growth and expansion of our pipeline. Excluding contributions from Kuvan, for which a generic became available during 2020, total revenues grew 13% in 2020, and generated $85 million of positive operating cash flows for the full year, underscoring the essential nature of our medicines."

Mr. Bienaimé continued, "The most recent Phase 3 data updates from our latest-stage development programs in achondroplasia and severe hemophilia A demonstrated significant efficacy. In the largest gene therapy trial ever conducted for the treatment of severe hemophilia A, we were pleased that valoctocogene roxaparvovec was the first in hemophilia A to demonstrate statistically significant evidence of annualized bleed rate superiority over standard of care recombinant FVIII. Based on these results, we are very encouraged that one infusion of valoctocogene roxaparvovec gene therapy may potentially address the high treatment burden for people with severe hemophilia A. We are targeting submission of the one-year Phase 3 results to the European Medicines Agency in the second quarter of 2021 and planning to dialog with the FDA to align on steps to obtain approval in the United States."

"Also in 2021, we look forward to the potential approval of vosoritide, which would be the first pharmacological treatment to address the underlying cause of achondroplasia, the most common form of dwarfism. We announced in the fourth quarter of 2020 that vosoritide demonstrated sustained growth effects for over two years in children with achondroplasia participating in our Phase 3 extension study. In addition to the large, Phase 3 program currently in the extension phase, we have built a multi-pronged dossier of additional studies to support our understanding of the unmet medical need for children with achondroplasia and the effects of vosoritide in this condition. In addition to the highly statistically significant placebo-controlled Phase 3 results, the program includes the 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. Many families are keen to seek early treatment for their children so we are hopeful that, if approved, vosoritide will become available later in 2021 upon potential approvals."

2021 Full-Year Financial Guidance (in millions, except %)

Item	2021 Guidance *		
Total Revenues	$1,750	to	$1,850
Vimizim Net Product Revenues	$570	to	$610
Kuvan Net Product Revenues	$250	to	$290
Naglazyme Net Product Revenues	$365	to	$395

Palynziq Net Product Revenues	$210	to	$250
Brineura Net Product Revenues	$120	to	$140
Cost of Sales (% of Total Revenues)	23%	to	25%
Research and Development Expense	$645	to	$695
Selling, General and Administrative Expense	$725	to	$775
GAAP Net Loss	($130)	to	($80)
Non-GAAP Income [(1)]	$170	to	$220

*2021 Guidance takes into consideration ongoing expected impact from the COVID-19 pandemic in 2021 assuming consistent trends experienced during 2020.

(1) 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 January 10, 2021, the Company announced positive top-line, one-year data results from its ongoing global Phase 3 GENEr8-1 study of valoctocogene roxaparvovec, an investigational gene therapy for the treatment of adults with severe hemophilia A. Data from the study in the pre-specified primary analysis for Annualized Bleeding Rate (ABR) showed that a single dose of valoctocogene roxaparvovec significantly reduced ABR by 84% compared with prior treatment with prophylactic FVIII infusions. These results were from a pre-specified group of participants in a non-interventional prospective baseline observational study (rollover population; N=112) with a median follow-up of 60.1 weeks after dosing with valoctocogene roxaparvovec. 80% of the rollover participants were bleed-free starting at week five after treatment.

  Additionally, at the end of the first year post-infusion with valoctocogene roxaparvovec, participants in the modified intent-to-treat (mITT) population (N=132) had a mean endogenous Factor VIII expression level of 42.9 (SD 45.5, median 23.9) IU/dL, as measured by the chromogenic substrate (CS) assay, supporting the marked clinical benefits observed with abrogation of bleeding episodes and Factor VIII infusion treatment rate. Factor VIII expression declined at a slower rate compared to the Phase 1/2 study, and remained in a range to provide hemostatic efficacy. In a subset of the mITT population that had been dosed at least two years prior to the data cut date (N=17), Factor VIII expression declined from a mean of 42.2 (SD 50.9, median 23.9) IU/dL at the end of year one to a mean of 24.4 (SD 29.2, median 14.7) IU/dL at the end of year two with continued hemostatic efficacy demonstrated by a mean ABR of 0.9 (median 0.0) bleeding episodes per year.

  Valoctocogene roxaparvovec also significantly reduced the mean annualized Factor VIII usage in the rollover population by 99% from 135.9 (median 128.6) to 2.0 (median 0.0) infusions per year (p-value <0.0001).

  In the U.S., 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 Company plans to meet with FDA to review the two-year data request and share the Phase 3 GENEr8-1 results announced on January 10, 2021. BioMarin is targeting submission of the Marketing Authorization Application (MAA) with these results to the EMA in the second quarter of 2021 pending confirmation in presubmission meetings.

- **Vosoritide for children with achondroplasia:** On December 21, 2020 the Company announced that children in the open-label long-term extension of the Phase 3 study of vosoritide, an investigational, once daily injection analog of C-type Natriuretic Peptide (CNP), maintained an increase in Annual Growth Velocity (AGV) through the second year of continuous treatment. An analysis, comparing all children randomized and treated with vosoritide for two years (n=52) to all children from the run-in study who were randomized to receive placebo with an untreated observation period of two years (n=38), showed improvement in one-year height change in the treated group relative to the untreated group that was similar in the second year of treatment, 1.79 cm, as in the first year of treatment, 1.73 cm. The cumulative height gain over the 2-year treatment period was 3.52 cm more than the untreated children.

  In 2020, marketing applications for vosoritide were validated and accepted by EMA and FDA, respectively. The CHMP opinion is expected in Europe in June of 2021. The U.S. New Drug Application (NDA) for vosoritide is under review by the FDA with a Prescription Drug User Fee Act (PDUFA) target action date of August 20, 2021.

  In January 2021, the Company received notice from FDA that the NDA for vosoritide had been granted Priority Review Designation. Under this designation, the vosoritide NDA may qualify for a Priority Review Voucher (PRV) upon approval. A PRV confers priority review to a subsequent drug application that would not otherwise qualify for that designation. The rare pediatric disease review voucher program is designed to encourage development of new drugs and biologics for the prevention or treatment of rare pediatric diseases.

- **Palynziq for 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 Palynziq (pegvaliase-pqpz) Injection for treatment of adults with 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 Palynziq.

  Palynziq is indicated to reduce blood Phe concentrations in adults with PKU, who have uncontrolled blood Phe concentrations greater than 600 µmol/L on existing management. Palynziq, 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 PKU:** The Company announced that it plans to dose escalate in PHEarless, the Phase 1/2 study of BMN 307 based on encouraging Phe lowering and safety signals observed in study participants who were treated with the lowest dose. 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 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 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.
- **BMN 255 for a subset of chronic renal disease:** On January 11, 2021 the Company announced that it filed an IND in 2020 for BMN 255, a small molecule for a subset of chronic renal disease. BMN 255 was driven by genetic discoveries for both mechanism and for identifying individuals for treatment.
- **BMN 351 for Duchenne Muscular Dystrophy (DMD):** IND-enabling studies are underway with BMN 351, an oligonucleotide therapy that has demonstrated a high-level of protein expression in experimental animals possessing

skippable dystrophic mutations and at doses that are promising in regard to safety. The Company intends to determine timing of a potential IND filing at the end of the year based on results of ongoing IND-enabling studies.

BioMarin will host a conference call and webcast to discuss fourth quarter and full-year 2020 financial results today, Thursday, February 25, 2021 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: 6488682	Conference ID: 6488682

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 Loss, Non-GAAP Income, and other specified income statement guidance for the full-year 2021; the timing of BioMarin's clinical development and commercial prospects, including announcements of data from clinical studies and trials; 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 for valoctocogene roxaparvovec 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 (iii) BioMarin's plans to meet with FDA to review the two-year data request and share the Phase 3 GENEr8-1 results announced on January 10, 2021, (iv) that the CHMP opinion for vosoritide is expected in Europe in the second half of 2021; and (v) the target PDUFA action date with respect to vosoritide of August 20, 2021; 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; and the expected benefits and availability of BioMarin's product candidates, including (i) that valoctocogene roxaparvovec gene therapy may potentially address the high treatment burden for people with severe hemophilia A and (ii) BioMarin's hope that, if approved, vosoritide will become available later in 2021.

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, including BioMarin's projected impact of the COVID-19 pandemic on its global revenue sources, including due to demand interruptions such as missed patient infusions and delayed treatment starts for new patients; 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 September 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 PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

December 31, 2020 and December 31, 2019

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

	December 31, 2020	December 31, 2019(1)
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 649,158	$ 437,446
Short-term investments	416,228	316,361
Accounts receivable, net	448,351	377,404
Inventory	698,548	680,275
Other current assets	129,934	130,657
Total current assets	2,342,219	1,942,143
Noncurrent assets:		
Long-term investments	285,473	411,978
Property, plant and equipment, net	1,032,471	1,010,868
Intangible assets, net	417,271	456,580
Goodwill	196,199	197,039
Deferred tax assets	1,432,150	549,422
Other assets	142,237	122,009
Total assets	$ 5,848,020	$ 4,690,039
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 492,548	$ 570,621
Short-term convertible debt, net	—	361,882
Total current liabilities	492,548	932,503

Noncurrent liabilities:		
Long-term convertible debt, net	1,075,145	486,238
Long-term contingent consideration	60,130	50,793
Other long-term liabilities	114,195	98,124
Total liabilities	$ 1,742,018	$ 1,567,658
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 181,740,999 and 179,838,114 shares issued and outstanding, respectively.	182	180
Additional paid-in capital	4,993,407	4,832,707
Company common stock held by Nonqualified Deferred Compensation Plan	(9,839)	(9,961)
Accumulated other comprehensive income	(16,139)	20,164
Accumulated deficit	(861,609)	(1,720,709)
Total stockholders' equity	4,106,002	3,122,381
Total liabilities and stockholders' equity	$ 5,848,020	$ 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

Three and Twelve Months Ended December 31, 2020 and 2019

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	**2019**	**2020**	**2019 (1)**
	(unaudited)	(unaudited)	(unaudited)	
REVENUES:				
Net product revenues	$ 437,045	$ 436,585	$ 1,805,861	$ 1,661,043
Royalty and other revenues	15,072	17,858	54,594	43,005
Total net revenues	452,117	454,443	1,860,455	1,704,048
OPERATING EXPENSES:				
Cost of sales	126,138	95,899	524,272	359,466
Research and development	156,667	172,812	628,116	715,007
Selling, general and administrative	195,512	187,900	737,669	680,924
Intangible asset amortization and contingent				

consideration	18,640	16,994	66,658	74,108
Gain on sale of nonfinancial assets	—	(10,000)	(59,495)	(25,000)
Total operating expenses	496,957	463,605	1,897,220	1,804,505
LOSS FROM OPERATIONS	(44,840)	(9,162)	(36,765)	(100,457)
Equity in the income (loss) of BioMarin/Genzyme LLC	1,071	193	(6)	(587)
Interest income	3,071	5,211	16,610	22,748
Interest expense	(4,749)	(6,930)	(29,309)	(23,460)
Other income, net	5,262	907	7,148	6,945
LOSS BEFORE INCOME TAXES	(40,185)	(9,781)	(42,322)	(94,811)
Benefit from income taxes	(62,284)	(24,805)	(901,422)	(70,963)
NET INCOME (LOSS)	22,099	15,024	859,100	(23,848)
NET INCOME (LOSS) PER SHARE, BASIC	$ 0.12	$ 0.08	$ 4.75	$ (0.13)
NET INCOME (LOSS) PER SHARE, DILUTED	$ 0.12	$ 0.08	$ 4.53	$ (0.13)
Weighted average common shares outstanding, basic	181,435	179,531	180,804	179,039
Weighted average common shares outstanding, diluted	184,476	182,412	191,678	179,039

(1) December 31, 2019 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, 2019, filed with the SEC on February 27, 2020.

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,		
	2020	2019	% Change	2020	2019 (1)	% Change
	(unaudited)	(unaudited)		(unaudited)		
PKU franchise	$ 138.6	$ 154.3	(10) %	$ 628.7	$ 550.3	14 %
Vimizim	142.5	132.3	8 %	544.4	544.3	— %
Naglazyme	119.7	94.8	26 %	391.3	374.3	5 %
Brineura	35.0	25.2	39 %	110.2	72.0	53 %
Firdapse [2]	—	6.1	(100) %	1.2	22.3	(95) %
Net Product Revenues Marketed by BioMarin	435.8	412.7		1,675.8	1,563.2	
Aldurazyme Net Product Revenues Marketed by Genzyme	1.2	23.9	(95) %	130.1	97.8	33 %

Total Net Product Revenues	~~$ 437.0~~	~~$ 436.6~~	~~$ 1,805.9~~	~~$ 1,661.0~~

(1) December 31, 2019 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, 2019, filed with the SEC on February 27, 2020.

(2) 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 twelve months ended months ending December 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,		
	2020	**2019**	**% Change**	**2020**	**2019 (1)**	**% Change**
	(unaudited)	(unaudited)		(unaudited)		
Kuvan	$ 89.0	122.6	(27) %	$ 457.7	463.4	(1) %
Palynziq	49.6	31.7	56 %	171.0	86.9	97 %
Total PKU franchise	$ 138.6	$ 154.3	(10) %	$ 628.7	$ 550.3	14 %

(1) December 31, 2019 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, 2019, filed with the SEC on February 27, 2020.

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
	2020	2019	2020	2019	December 31, 2021
GAAP Net Income (Loss)	**$ 22.1**	**$ 15.0**	**$ 859.1**	**$ (23.8)**	**$ (130.0) — $ (80.0)**
Interest expense, net	1.7	1.7	12.7	0.7	11.0 — 11.0
Benefit from income taxes	(62.3)	(24.8)	(901.4)	(71.0)	(16.0) — (16.0)
Depreciation expense	11.9	9.5	43.0	51.8	48.0 — 48.0
Amortization expense	15.4	16.3	62.2	53.5	62.0 — 62.0
Stock-based compensation expense	47.5	38.0	189.7	159.8	186.0 — 186.0
Contingent consideration expense	3.2	0.7	4.5	20.6	9.0 — 9.0
Provision for inventory reserve, net [1]	—	—	75.6	—	— — —
Gain on sale of nonfinancial assets	—	(10.0)	(59.5)	(25.0)	— — —
Licensed In-Process R&D [2]	—	—	26.3	—	— — —
Non-GAAP Income	$ 39.5	$ 46.4	$ 312.2	$ 166.6	$ 170.0 — $ 220.0

(1) Represents a $81.2 million charge related to pre-launch valoctocogene roxaparvovec 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 December 31,						
	2020				2019		
		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-Base Compensatic Contingent Considerati and Other Adjustment
Cost of sales	**$ 126.1**	$ —	$ (5.9)	$ 120.2	**$ 95.9**	$ —	$ (3.5)
Research and development	**156.7**	(6.7)	(16.5)	133.5	**172.8**	(3.8)	(13.5)
Selling, general and administrative	**195.5**	(5.2)	(25.1)	165.2	**187.9**	(5.7)	(21.0)
Intangible asset amortization and contingent consideration	**18.6**	(15.4)	(3.2)	—	**17.0**	(16.3)	(0.7)
Gain on sale of nonfinancial assets	**—**	—	—	—	**(10.0)**	—	10.0
Interest expense, net	**(1.7)**	1.7	—	—	**(1.7)**	1.7	—
Benefit from income taxes	**(62.3)**	62.3	—	—	**(24.8)**	24.8	—
GAAP Net Income /Non-GAAP Income	**$ 22.1**	$ (33.3)	$ 50.7	$ 39.5	**$ 15.0**	$ 2.7	$ 28.7

	Twelve months ended December 31,

	2020				2019		
		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-Bas Compensati Continge Considerat and Othe Adjustmer
Cost of sales	**$ 524.3**	$ —	$ (101.9)	$ 422.4	**$ 359.5**	$ —	(16.1)
Research and development	**628.1**	(21.9)	(88.2)	518.0	**715.0**	(26.9)	(56.6)
Selling, general and administrative	**737.7**	(21.1)	(101.5)	615.1	**680.9**	(24.9)	(87.1)
Intangible asset amortization and contingent consideration	**66.7**	(62.2)	(4.5)	—	**74.1**	(53.5)	(20.6)
Gain on sale of nonfinancial assets	**(59.5)**	—	59.5	—	**(25.0)**	—	25.0
Interest expense, net	**(12.7)**	12.7	—	—	**(0.7)**	0.7	—
Benefit from income taxes	**(901.4)**	901.4	—	—	**(71.0)**	71.0	—
GAAP Net Income (Loss)/Non-GAAP Income	**859.1**	(783.5)	236.6	312.2	**(23.8)**	35.0	155.4

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