BioMarin Announces First Quarter 2020 Total Revenue Growth of 25% to $502 million

- U.S. Marketing Application for Valoctocogene Roxaparvovec for Severe Hemophilia A Accepted by FDA under Priority Review with Prescription Drug User Fee Act (PDUFA) action date of August 21, 2020

- European Marketing Application for Valoctocogene Roxaparvovec for Severe Hemophilia A Accepted by EMA; Committee for Medicinal Products for Human Use (CHMP) Opinion Expected by Late 2020/Early 2021

- Regulatory Submissions Planned for Marketing Authorization of Vosoritide to Treat Children with Achondroplasia in Third Quarter of 2020 in both the U.S. and Europe

- For the Full-Year 2020 BioMarin Continues to Expect to be Profitable on a GAAP Basis for the First Time with no Guidance Change to either GAAP Net Income or Non-GAAP Income

- 2020 Full-Year Total Revenue Guidance Reduced by 5% Due to Anticipated Impact of the Coronavirus (COVID-19) Pandemic Assuming Return to Normalized Demand Patterns in the Second Half of 2020

SAN RAFAEL, Calif., April 29, 2020 /PRNewswire/ --

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

	Three Months Ended March 31,		
	2020	**2019**	**% Change**
Total Revenues	$ 502.1	$ 400.7	25 %
Net Product Revenues Marketed by BioMarin (1)	433.3	349.2	24 %
Vimizim Net Product Revenues	137.2	125.8	9 %
Kuvan Net Product Revenues	122.0	106.9	14 %
Naglazyme Net Product Revenues	114.3	86.9	32 %
Palynziq Net Product Revenues	34.6	12.3	181 %
Brineura Net Product Revenues	24.0	12.2	97 %
Aldurazyme Net Product Revenues	55.7	45.3	23 %
GAAP Net Income (Loss)	$ 81.4	$ (56.5)	
GAAP Net Income (Loss) per Share – Basic	$ 0.45	$ (0.32)	
GAAP Net Income (Loss) per Share – Diluted	$ 0.44	$ (0.32)	
Non-GAAP Income (2)	$ 116.5	$ 24.8	

	March 31, 2020	**December 31, 2019**
Cash, cash equivalents and investments	$ 1,149.2	$ 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 9 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 will be reflected in the Company's consolidated financial statements for the three months ending March 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, 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 first quarter ended March 31, 2020.

Total Revenues increased 25% to $502.1 million. The increase in Total Revenues was primarily attributed to increased Net Product Revenues which were $489.0 million in the first quarter of 2020, compared to $394.5 million for the first quarter of 2019. The increase in Net Product Revenues was attributed to the following:

- Naglazyme Net Product Revenues increased by $27.4 million, or 32%, primarily due to orders from Russia and Brazil;
- Palynziq Net Product Revenues increased by $22.3 million or 181%, driven by combination of revenue from U.S. patients achieving maintenance dosing and new patients initiating therapy;
- Kuvan Net Product Revenues increased by $15.1 million, or 14%, primarily driven by patient growth in North America;
- Brineura Net Product Revenues increased by $11.8 million, or 97%, due in large part to global patient growth;
- Vimizim Net Product Revenues increased by $11.4 million, or 9%, driven primarily due to orders from Brazil; and
- Aldurazyme Net Product Revenues increased $10.4 million, or 23%, due to higher sales volume to Sanofi Genzyme.

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

- increased gross profits of $79.1 million primarily driven by increased product sales;
- a net gain on the sale of nonfinancial assets of $59.5 million due to the divestiture and sale of the Firdapse business; and
- decreased research and development (R&D) expenses; partially offset by
- higher selling, general and administrative (SG&A) expense related to pre-commercialization activities for valoctocogene roxaparvovec, commercialization activities in support of the EU commercial launch and continued U.S. expansion of Palynziq, and foreign currency exchange losses.

Non-GAAP Income for the first quarter of 2020 increased to $116.5 million, compared to Non-GAAP Income of $24.8 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 higher gross profit and decreased R&D expense, partially offset by higher SG&A expense.

As of March 31, 2020, BioMarin had cash, cash equivalents and investments totaling approximately $1.1 billion, as compared to $1.2 billion on December 31, 2019.

Commenting on first quarter 2020 results, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "With the arrival of COVID-19 to the many regions where we do business, BioMarin employees performed in unprecedented ways to ensure the continued supply of our critically-important medicines to the people we serve. I am proud of the commitment and dedication demonstrated by our colleagues in these challenging times. Our strong financial results in the first quarter underscore both the essential-nature of our products to patients and the extraordinary efforts made to maintain supply around the world. In the face of the many challenges of COVID-19, our regulatory team further progressed our next two potential commercial products. The Biologics License Application (BLA) for valoctocogene roxaparvovec for severe hemophilia A was accepted for Priority Review from the FDA with an action date of August 21, 2020. This milestone represents a tremendous achievement for BioMarin, but the potential approval of the first gene therapy in any type of hemophilia is an even greater triumph for the hemophilia community. They have been waiting decades for this groundbreaking advancement and we are honored to be on this journey together. With an approval decision for valoctocogene roxaparvovec expected later this year, our commercial team prepares eagerly to launch what we believe is the most innovative product yet for people with bleeding disorders."

Mr. Bienaimé continued, "Based on positive interactions with U.S. and European regulatory authorities in the quarter, we plan to submit marketing applications in both regions for vosoritide to treat children with achondroplasia in the third quarter of this year. Our multi-pronged dossier of data encompasses long-term clinical results in 5 to 18 year-olds, natural history data, the ongoing study of newborns through 5 years, and highly statistically significant placebo-controlled Phase 3 results. The positive and significant results from our vosoritide clinical programs have led us to believe that this potential drug could be the first pharmacological treatment for the underlying cause of achondroplasia. Interest in our clinical studies with vosoritide has been extremely robust, demonstrating that families are keen to seek early treatment for their children."

Mr. Bienaimé concluded, "2020 is expected to be a transformational year for BioMarin, despite impact from COVID-19 in the near-term. The agility demonstrated by BioMarin employees in the face of this global pandemic has enabled the continued supply of our essential medicines to the patients who need them. And while we expect minor financial impact in the near-term, our business is well-positioned to weather such challenges. Our first quarter revenue growth and improvement in profitability support our belief that 2020 continues to look poised to be one of our most significant value-creating years to date."

2020 Full-Year Financial Guidance

Due to the uncertainty surrounding the COVID-19 pandemic and the potential impact on its business, BioMarin is reducing its guidance for Total Revenues and Net Product Revenues for Vimizim, Naglazyme and Palynziq for 2020.

Item	Provided February 26, 2020			Updated April 29, 2020		
Total Revenues [(1)]	$1,950	to	$2,050	$1,850	to	$1,950
Vimizim Net Product Revenues	$560	to	$610	$530	to	$570
Kuvan Net Product Revenues	$430	to	$480	Unchanged		
Naglazyme Net Product Revenues	$380	to	$420	$360	to	$400
Palynziq Net Product Revenues	$180	to	$210	$160	to	$190
Brineura Net Product Revenues	$85	to	$115	Unchanged		

Cost of Sales (% of Total Revenues)	20	%	to	21	%	Unchanged
Research and Development Expense	$675		to	$725		Unchanged
Selling, General and Administrative Expense	$780		to	$830		Unchanged
GAAP Net Income [(2)]	$20		to	$80		Unchanged
Non-GAAP Income [(3)]	$260		to	$310		Unchanged

(1) Updated 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. The updated revenue guidance assumes stabilization of such interruptions in the second half of 2020.

(2) 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.

(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 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:** The FDA review of the BLA, under Priority Review, for valoctocogene roxaparvovec is on-track with a PDUFA action date of 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. Although the MAA remains under accelerated assessment at this time, the Company expects the review procedure to be extended by at least 3 months due to COVID-19 delays. Further, the Company believes there is a high possibility that the MAA will revert to the standard review procedure, as is the case with most filings that initially receive accelerated assessment. Because of the combination of these events, the Company expects an opinion from the CHMP in late 2020/early 2021.

  The Company recently received EMA licensure of its gene therapy manufacturing facility for the production of valoctocogene roxaparvovec, an important step in obtaining regulatory approval of the product in the EU. The Health Products Regulatory Authority (HPRA) of Ireland conducted, on behalf of EMA, a pre-approval inspection in the first quarter and issued a cGMP certification in the second quarter. The inspection of the facility by FDA is expected to be complete during the second quarter, which would allow for potential licensure of the facility in the U.S. consistent with the August 21st PDUFA date.

  The marketing applications are based on the Phase 3 interim analysis and the updated three-year Phase 1/2 data from patients treated with valoctocogene roxaparvovec. The Company believes that 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 plan 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 four-year update with the 6e13 vg/kg dose subjects and a three-year update with the 4e13 vg/kg dose subjects from the ongoing Phase 2 study in mid-2020.

- **Vosoritide for children with achondroplasia:** On April 6, 2020, the Company announced that based on recent meetings with health authorities in the U.S. and Europe, it plans to submit marketing applications to the FDA and EMA in the third quarter of 2020. The marketing applications are based on 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.

  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 of treatment 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, and has completed enrollment. Completion of enrollment of cohort 3, which includes children ages 0 to 6 months old, is expected to be somewhat delayed due to impact from COVID-19.

- **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 impact of COVID-19 has created uncertainty about when it will be safe for patients to be dosed in PHEARLESS, our Phase 1/2 study of BMN 307. The Company currently estimates that dosing will begin in the second half of 2020. In the meantime, new sites are currently being prepared to open and enroll patients. All subjects participating in the PHEARLESS study will receive product made at commercial scale from BioMarin's award-winning gene therapy manufacturing facility. Both the FDA and EMA have granted BMN 307 Orphan Drug 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 announced its third gene therapy candidate, BMN 331, for the treatment of Hereditary Angioedema (HAE). BioMarin plans to build on its ever wider and deeper expertise in developing gene therapies for severe hemophilia A and PKU to improve efficiencies in the development process, and to optimize capsid and transgene design. The Company is monitoring developments surrounding COVID-19 but expects to begin IND-enabling studies in 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 is monitoring developments surrounding COVID-19 but currently expects the trial with vosoritide for DISS to begin in the second half of 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 first quarter 2020 financial results today, Wednesday, April 29, 2020 at 4:15 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: 833.360.0852	Replay Dial-in Number: 855.859.2056
International Dial-in Number: 630.652.5841	Replay International Dial-in Number: 404.537.3406
Conference ID: 6194595	Conference ID: 6194595

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 (i) BioMarin's expectation of a minor financial impact in the near term, (ii) BioMarin's business being well positioned to weather such challenges (iii) that BioMarin will become profitable on a GAAP basis for the first time in 2020 and (iv) that the COVID-19-related demand interruptions such as missed patient infusions and delayed treatment starts for new patients will stabilize in the second half of 2020; 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 second half 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 the second half of 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 submit marketing applications for vosoritide for children with achondroplasia in the third quarter of 2020, (ii) the productivity, capacity and licensure of its gene therapy manufacturing facility, including the timing of such licensure, and (iii) the potential approval and commercialization of BioMarin's product candidates, including 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 Annual Report on Form 10-K for the year ended December 31, 2019 as such factors may be updated by any subsequent reports. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise.

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

March 31, 2020 and December 31, 2019

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

	March 31, 2020	December 31, 2019 (1)
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 476,632	$ 437,446
Short-term investments	381,764	316,361
Accounts receivable, net	396,384	377,404
Inventory	705,652	680,275
Other current assets	155,817	130,657
Total current assets	2,116,249	1,942,143
Noncurrent assets:		

Long-term investments	290,796	411,978
Property, plant and equipment, net	1,009,972	1,010,868
Intangible assets, net	443,717	456,580
Goodwill	196,199	197,039
Deferred tax assets	539,990	549,422
Other assets	125,918	122,009
Total assets	$ 4,722,841	$ 4,690,039
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 454,506	$ 570,621
Short-term convertible debt, net	365,964	361,882
Total current liabilities	820,470	932,503
Noncurrent liabilities:		
Long-term convertible debt, net	486,713	486,238
Long-term contingent consideration	50,524	50,793
Other long-term liabilities	125,172	98,124
Total liabilities	$ 1,482,879	$ 1,567,658
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 180,761,969 and 179,838,114 shares issued and outstanding, respectively.	181	180
Additional paid-in capital	4,854,814	4,832,707
Company common stock held by Nonqualified Deferred Compensation Plan	(9,832)	(9,961)
Accumulated other comprehensive income	34,127	20,164
Accumulated deficit	(1,639,328)	(1,720,709)
Total stockholders' equity	3,239,962	3,122,381
Total liabilities and stockholders' equity	$ 4,722,841	$ 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 Months Ended March 31, 2020 and 2019

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

	Three Months Ended March 31,

	2020 (unaudited)	2019 (unaudited)
REVENUES:		
Net product revenues	$ 489,043	$ 394,483
Royalty and other revenues	13,026	6,262
Total net revenues	502,069	400,745
OPERATING EXPENSES:		
Cost of sales	111,374	89,182
Research and development	142,257	183,591
Selling, general and administrative	187,295	162,158
Intangible asset amortization and contingent consideration	15,677	19,765
Gain on sale of nonfinancial assets	(59,495)	—
Total operating expenses	397,108	454,696
INCOME (LOSS) FROM OPERATIONS	104,961	(53,951)
Equity in the loss of BioMarin/Genzyme LLC	(77)	(185)
Interest income	5,244	6,298
Interest expense	(6,915)	(6,727)
Other income, net	(1,861)	1,608
INCOME (LOSS) BEFORE INCOME TAXES	101,352	(52,957)
Provision for income taxes	19,971	3,516
NET INCOME (LOSS)	81,381	(56,473)
NET INCOME (LOSS) PER SHARE, BASIC	$ 0.45	$ (0.32)
NET INCOME (LOSS) PER SHARE, DILUTED	$ 0.44	$ (0.32)
Weighted average common shares outstanding, basic	179,898	178,271
Weighted average common shares outstanding, diluted	187,163	178,271

The following table presents Net Product Revenues by Product:

Net Product Revenues by Product

(In millions of U.S. dollars)

	Three Months Ended March 31,		
	2020 (unaudited)	2019 (unaudited)	% Change
Brineura	$ 24.0	$ 12.2	97 %
Firdapse [1]	1.2	5.1	(76) %
Naglazyme	114.3	86.9	32 %

PKU franchise	156.6	119.2	31 %
Vimizim	137.2	125.8	9 %
Net Product Revenues Marketed by BioMarin	433.3	349.2	
Aldurazyme Net Product Revenues Marketed by Genzyme	55.7	45.3	23 %
Total Net Product Revenues	$ 489.0	$ 394.5	

(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 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 March 31,		
	2020	**2019**	**% Change**
	(unaudited)	(unaudited)	
Kuvan	$ 122.0	106.9	14 %
Palynziq	34.6	12.3	181 %
Total PKU franchise	$ 156.6	$ 119.2	31 %

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 March 31,		Guidance Year Ending		
	2020	2019	December 31, 2020		
GAAP Net Income (Loss)	**$ 81.4**	**$ (56.5)**	**$ 20.0**	**—**	**$ 80.0**
Interest expense, net	1.7	0.4	3.0	—	2.0
Provision for income taxes	20.0	3.5	4.5	—	10.5
Depreciation expense	10.3	14.9	50.0	—	47.0
Amortization expense	15.7	7.5	63.0	—	61.0
Stock-based compensation expense	46.9	42.7	177.0	—	167.0
Contingent consideration expense	—	12.3	2.0	—	2.0
Gain on sale of nonfinancial assets	(59.5)	—	(59.5)	—	(59.5)
Non-GAAP Income	$ 116.5	$ 24.8	$ 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 March 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 Compensat Contingе Considerat and Othe Adjustme
Cost of sales	**$ 111.4**	$ —	$ (5.1)	$ 106.3	**$ 89.2**	$ —	$ (4.
Research and development	**142.3**	(4.8)	(13.7)	123.8	**183.6**	(9.3)	(13.
Selling, general and administrative	**187.3**	(5.5)	(28.1)	153.7	**162.2**	(5.6)	(24.
Intangible asset amortization and contingent consideration	**15.7**	(15.7)	—	—	**19.8**	(7.5)	(12.
Gain on sale of nonfinancial assets	**(59.5)**	—	59.5	—	**—**	—	-
Interest expense, net	**(1.7)**	1.7	—	—	**(0.4)**	0.4	-
Provision for income taxes	**20.0**	(20.0)	—	—	**3.5**	(3.5)	-
GAAP Net Income (Loss)/Non-GAAP Income	**$ 81.4**	$ 47.7	$ (12.6)	$ 116.5	**$ (56.5)**	$ 26.3	$ 55.

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https://investors.biomarin.com/2020-04-29-BioMarin-Announces-First-Quarter-2020-Total-Revenue-Growth-of-25-to-502-million