BioMarin InvestorRoom

BioMarin Announces Second Quarter 2020 Total Revenue Growth of 11% to $430 million

- Impact of the Coronavirus (COVID-19) Pandemic as Expected in the Second Quarter; 2020 Full-Year Revenue Guidance Reaffirmed Assuming Demand Patterns Return to Normal in Late 2020
- U.S. Marketing Application for Valoctocogene Roxaparvovec for Severe Hemophilia A under Priority Review by Food and Drug Administration (FDA) with Prescription Drug User Fee Act (PDUFA) Target Action Date of August 21, 2020
- Marketing Authorization Application (MAA) for Valoctocogene Roxaparvovec for Severe Hemophilia A Validated by the European Medicines Agency (EMA); Committee for Medicinal Products for Human Use (CHMP) Opinion Expected by Late 2020/Early 2021
- MAA Submitted to EMA for Vosoritide to Treat Children with Achondroplasia July 23; On track to Submit a New Drug Application (NDA) to the FDA Late in the Third Quarter of 2020
- For the Full-Year 2020 BioMarin Continues to Expect to be Profitable on a GAAP Basis for the First Time

SAN RAFAEL, Calif., Aug. 4, 2020 /PRNewswire/ --

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

	Three Months Ended June 30,			Six Months Ended June 30,		
	2020	2019	% Change	2020	2019	% Change
Total Revenues	$ 429.5	$ 387.8	11 %	$ 931.6	$ 788.5	18 %
Net Product Revenues Marketed by BioMarin [1]	386.8	373.3	4 %	820.1	722.5	14 %
Vimizim Net Product Revenues	116.7	122.7	(5) %	253.9	248.5	2 %
Kuvan Net Product Revenues	122.6	113.3	8 %	244.6	220.2	11 %
Naglazyme Net Product Revenues	81.0	98.2	(18) %	195.3	185.1	6 %
Palynziq Net Product Revenues	40.7	18.8	116 %	75.3	31.1	142 %
Brineura Net Product Revenues	25.8	14.8	74 %	49.8	27.0	84 %
Aldurazyme Net Product Revenues	32.3	5.8	457 %	88.0	51.1	72 %
GAAP Net Income (Loss)	$ (29.2)	$ (37.4)		$ 52.2	$ (93.9)	
GAAP Net Income (Loss) per Share – Basic	$ (0.16)	$ (0.21)		$ 0.29	$ (0.53)	
GAAP Net Income (Loss) per Share – Diluted	$ (0.16)	$ (0.21)		$ 0.28	$ (0.53)	
Non-GAAP Income [2]	$ 57.4	$ 17.1		$ 173.9	$ 42.2	

	June 30, 2020	December 31, 2019
Cash, cash equivalents and investments	$ 1,703.4	$ 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 is reflected in the Company's consolidated financial statements for the three and six months ending June 30, 2020; as a result of the transaction BioMarin will not recognize Net Product Revenues from Firdapse in the future.

(2) Non-GAAP Income is defined by the Company as reported GAAP Net Income, excluding net interest expense, provision for (benefit from) income taxes, depreciation expense, amortization expense, stock-based compensation expense, contingent consideration expense and, in certain periods, certain other specified items. Refer to Non-GAAP Information beginning on page 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 second quarter ended June 30, 2020.

Net Product Revenues for the second quarter of 2020 increased to $419.0 million, compared to $379.1 million in the second quarter of 2019. The increase in Net Product Revenues was attributed to the following:

- Aldurazyme Net Product Revenues increased by $26.5 million due to higher sales volume to Genzyme;
- Palynziq Net Product Revenues increased by $21.9 million driven by a combination of revenue from U.S. patients achieving maintenance dosing and new patients initiating therapy;
- Brineura Net Product Revenues increased by $11.0 million due in large part growth in the number of patients in all regions; and

- Kuvan Net Product Revenues increased by $9.3 million driven primarily by a U.S. price increase and Kuvan product mix; partially offset by
- Naglazyme and Vimizim Net Product Revenues combined decreased by $23.2 million primarily due to timing of orders as well as the impact of missed infusions resulting from the COVID-19 pandemic.

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

- an increase in gross profit (Total Revenues less Cost of Sales) of $21.2 million primarily driven by higher product sales; and
- an increase in the benefit from income taxes; partially offset by
- the effect of the one-time gain recognized in the second quarter of 2019 due to a third party's achievement of a commercial milestone related to previously sold intangible asset; and
- higher selling, general and administrative (SG&A) expense related to pre-commercialization activities for valoctocogene roxaparvovec.

Non-GAAP Income for the second quarter of 2020 increased to $57.4 million, compared to Non-GAAP Income of $17.1 million for the same period in 2019. The increase in Non-GAAP Income for the quarter, compared to the same period in 2019, was attributed to decreased R&D expense and higher gross profit, partially offset by higher SG&A expense.

As of June 30, 2020, BioMarin had cash, cash equivalents and investments totaling approximately $1.7 billion, as compared to $1.2 billion on December 31, 2019.

Commenting on second quarter 2020 results, Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin, said, "In the second quarter, BioMarin employees worked collaboratively to ensure access to our critically-important medicines to the people we serve, despite the global impact of COVID-19. In these challenging times, our strong financial results underscore both the essential nature of our products to patients and our ongoing efforts to maintain supply around the world."

Mr. Bienaimé continued, "In the second quarter at the World Federation of Hemophilia Virtual Congress, we were pleased to share the four-year data update from our ongoing Phase 1/2 study, which demonstrated sustained clinical benefit following a single administration of valoctocogene roxaparvovec. All participants in the study received a single administration of valoctocogene roxaparvovec in 2016 and remained off exogenous factor prophylaxis through year four. These data strengthen our confidence in valoctocogene roxaparvovec and the opportunity to address the unmet therapeutic needs of people with severe hemophilia A. With our marketing applications under review in both the United States and Europe, we await the potential approval of valoctocogene roxaparvovec. We believe each of the submissions represent the first time a gene therapy product for any type of hemophilia indication is under review by health authorities. With the outcome of the Priority Review of our BLA anticipated August 21, 2020, our commercial team is preparing to launch what we believe is the most innovative product yet for people with bleeding disorders."

"Another key milestone in the third quarter of this year, representing the culmination of years of clinical study and development, was the July 23 submission of a MAA to the EMA for vosoritide to treat children with achondroplasia. The company remains on track to submit a NDA to the FDA later in the third quarter. Our multi-pronged dossier of data encompasses long-term clinical results in 5 to 18 year-olds from our Phase 2 study, natural history data, the ongoing study of newborns through 5 years, and highly statistically significant placebo-controlled Phase 3 results. The positive results from our vosoritide clinical programs bolster our confidence in the potential for this drug to 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, "Despite impact from COVID-19 on our business in the short-term, we remain focused on working towards significant achievements that we believe will drive long-term value. Key milestones for the second half of 2020 include reaching GAAP profitability for a full year for the first time in our history, the potential approval of valoctocogene roxaparvovec, and the pursuit of vosoritide approval. With these exciting possibilities on the horizon, 2020 has the potential to be the most momentous year in our 20-year history."

2020 Full-Year Financial Guidance

GAAP Net Income guidance for 2020 has been updated to include the potential impact of intangible asset transfers between BioMarin entities. These intangible asset transfers are expected to occur in the second half of 2020, and are estimated to result in a one-time, non-cash income tax benefit of approximately $700 million to $900 million. The range acknowledges that the intangible asset transfers have not yet been completed and therefore the final value cannot yet be determined with certainty. The final valuation will be completed when the transactions occur. As a result, full year GAAP net income guidance has been updated to be in the range between $720 million and $980 million. The intangible asset transfers are not expected to impact Non-GAAP income.

Item	Provided April 29, 2020			Revised August 4, 2020		
Total Revenues (1)	$1,850	to	$1,950	Unchanged		
Vimizim Net Product Revenues	$530	to	$570	Unchanged		
Kuvan Net Product Revenues	$430	to	$480	Unchanged		
Naglazyme Net Product Revenues	$360	to	$400	Unchanged		
Palynziq Net Product Revenues	$160	to	$190	Unchanged		
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	$ 20	to	$ 80	$ 720	to	$ 980
Non-GAAP Income (2)	$260	to	$310	Unchanged		

(1) Revenue guidance reflects BioMarin's projected impact of the COVID-19 pandemic on its global revenue sources, mostly in the form of demand interruptions such as missed patient infusions and delayed treatment starts for new patients. The revenue guidance assumes stabilization of such interruptions in late 2020.

(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 June 17, 2020, the Company provided 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 a late-breaking oral presentation at World Federation of Hemophilia Virtual Summit. The results from both dose cohorts demonstrated that all subjects had remained off prophylactic Factor VIII treatment since receiving their single dose of valoctocogene roxaparvovec. In addition, cumulative mean annualized bleed rates (ABR) remained less than one in both cohorts and below pre-treatment baseline levels. In the six study participants who were previously on Factor VIII prophylaxis in the 6e13 vg/kg cohort, the data showed substantial and sustained reductions in bleeding that required Factor VIII infusions. During the four years following treatment with valoctocogene roxaparvovec, the cumulative mean ABR was 0.8, which represents a 95% reduction from baseline. In the fourth year, the mean ABR was 1.3 and the median was zero. There was a 96% reduction in mean Factor VIII usage to 5.4 infusions per year cumulatively over four years from the baseline of 135.6 infusions per year on Standard of Care prophylaxis.
- Similarly, in the six study participants in the 4e13 vg/kg cohort, the data showed substantial and sustained reductions in bleeding requiring Factor VIII infusions following treatment with valoctocogene roxaparvovec. The cumulative mean ABR was reduced by 93% to 0.9 with continued absence of target joint bleeds in 5 of 6 subjects during the three years observed, which represents a 93% reduction from baseline. During the third year of follow-up, the mean ABR was 0.5 and the median ABR was zero, and 67% or four out of six, of the study participants were bleed-free. Five out of six participants had no spontaneous bleeds. There was a 96% reduction in mean Factor VIII usage to 5.7 infusions per year cumulatively over three years from the baseline of 142.8 infusions per year. The FDA review of the BLA, under Priority Review, for valoctocogene roxaparvovec is on-track with a PDUFA target action date of August 21, 2020. In Europe, the MAA filing remains under accelerated assessment at this time. However, as communicated on the first quarter results call, the review procedure was extended by at least 3 months due to COVID-19 delays. Further, as is the case with most filings that initially receive accelerated assessment, there is a high possibility that the MAA will revert to a standard review procedure from accelerated assessment. Based on these assumptions, the CHMP opinion is expected in late 2020/early 2021.
- **Vosoritide for children with achondroplasia:** On July 23, 2020, the Company announced that it had submitted the MAA for vosoritide for the treatment of children with achondroplasia to the EMA. Vosoritide is an investigational, once daily injection of an analog of C-type Natriuretic Peptide (CNP). BioMarin plans to submit a vosoritide marketing application to the FDA later in the third quarter of 2020.

  The applications include 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 ($p<0.0001$). An ongoing, open-label, Dose Finding Phase 2 study of vosoritide for achondroplasia 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 natural history achondroplasia dataset (N=619). 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 mild-to-moderate injection site reactions being the most frequent adverse event; there were no clinically significant blood pressure decreases.

- **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.

  Depending on the ongoing impact of COVID-19, the Company currently believes that dosing in Phearless, the Phase 1/2 study of BMN 307, could begin later in the third quarter. In the meantime, sites are 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.

- **DiNA-001 for MYBPC3 hypertrophic cardiomyopathy (HCM):** On May 3, 2020 the Company announced that it had entered into a preclinical collaboration and license agreement with DiNAQOR AG (DiNAQOR), a gene therapy platform company, to develop novel gene therapies to treat rare genetic cardiomyopathies. DiNAQOR will receive 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 331 gene therapy product candidate for Hereditary Angioedema (HAE)** : The Company began IND-enabling studies in July with its third gene therapy candidate, BMN 331, for the treatment of Hereditary Angioedema (HAE). BioMarin plans to leverage its broad expertise in developing gene therapies for severe hemophilia A and PKU to improve efficiencies in the development process of BMN 331.
- **Vosoritide for the treatment of Genetic Causes of Short Stature (GSS):** In July, an investigator-initiated trial with vosoritide for GSS began enrolling participants. As previously announced, the Company plans to study vosoritide for treatment of broader genetic statural abnormalities starting with genetic short stature (GSS). This study is part of a research collaboration with Children's National Hospital in Washington, D.C.

BioMarin will host a conference call and webcast to discuss second quarter 2020 financial results today, Tuesday, August 4, 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: 3285215	Conference ID: 3285215

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 our belief that 2020 has the potential to be the most momentous year in BioMarin's 20-year history; the financial performance of BioMarin as a whole, including (i) that BioMarin expects to become profitable on a GAAP basis for the first time in 2020 and (ii) that the COVID-19-related demand interruptions will normalize in late 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 GSS, (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; the clinical development and commercialization of BioMarin's product candidates and commercial products, including (i) BioMarin's remaining on track to submit an NDA to the FDA later in the third quarter for vosoritide for children with achondroplasia ,and (ii) 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-Q for the quarter ended March 31, 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

June 30, 2020 and December 31, 2019

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

	June 30, 2020	December 31, 2019(1)
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 818,900	$ 437,446
Short-term investments	561,472	316,361
Accounts receivable, net	381,215	377,404
Inventory	743,852	680,275
Other current assets	142,127	130,657
Total current assets	2,647,566	1,942,143
Noncurrent assets:		
Long-term investments	323,058	411,978
Property, plant and equipment, net	1,010,917	1,010,868
Intangible assets, net	433,381	456,580
Goodwill	196,199	197,039
Deferred tax assets	555,137	549,422
Other assets	135,852	122,009
Total assets	$ 5,302,110	$ 4,690,039
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 443,202	$ 570,621
Short-term convertible debt, net	370,100	361,882
Total current liabilities	813,302	932,503
Noncurrent liabilities:		
Long-term convertible debt, net	1,073,202	486,238
Long-term contingent consideration	50,216	50,793
Other long-term liabilities	128,711	98,124
Total liabilities	$ 2,065,431	$ 1,567,658
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 181,148,332 and 179,838,114 shares issued and outstanding, respectively.	181	180
Additional paid-in capital	4,885,637	4,832,707
Company common stock held by Nonqualified Deferred Compensation Plan	(10,678)	(9,961)
Accumulated other comprehensive income	30,050	20,164
Accumulated deficit	(1,668,511)	(1,720,709)
Total stockholders' equity	3,236,679	3,122,381
Total liabilities and stockholders' equity	$ 5,302,110	$ 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

Six Months Ended June 30, 2020 and 2019

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	**2019**	**2020**	**2019**
	(unaudited)	(unaudited)	(unaudited)	(unaudited)
REVENUES:				
Net product revenues	$ 419,032	$ 379,075	$ 908,075	$ 773,558
Royalty and other revenues	10,453	8,688	23,479	14,950
Total net revenues	429,485	387,763	931,554	788,508
OPERATING EXPENSES:				
Cost of sales	97,967	77,436	209,341	166,618
Research and development	182,139	185,641	324,396	369,232
Selling, general and administrative	175,412	160,754	362,707	322,912
Intangible asset amortization and contingent consideration	14,912	20,286	30,589	40,051
Gain on sale of nonfinancial assets	—	(15,000)	(59,495)	(15,000)
Total operating expenses	470,430	429,117	867,538	883,813
INCOME (LOSS) FROM OPERATIONS	(40,945)	(41,354)	64,016	(95,305)
Equity in the loss of BioMarin/Genzyme LLC	(79)	(44)	(156)	(229)
Interest income	4,291	5,899	9,535	12,197
Interest expense	(8,048)	(6,866)	(14,963)	(13,593)
Other income, net	2,508	470	647	2,078
INCOME (LOSS) BEFORE INCOME TAXES	(42,273)	(41,895)	59,079	(94,852)
Provision for (benefit from) income taxes	(13,090)	(4,460)	6,881	(944)
NET INCOME (LOSS)	(29,183)	(37,435)	52,198	(93,908)
NET INCOME (LOSS) PER SHARE, BASIC	$ (0.16)	$ (0.21)	$ 0.29	$ (0.53)
NET INCOME (LOSS) PER SHARE, DILUTED	$ (0.16)	$ (0.21)	$ 0.28	$ (0.53)
Weighted average common shares outstanding, basic	180,729	179,048	180,314	178,662
Weighted average common shares outstanding, diluted	180,729	179,048	184,344	178,662

The following table presents Net Product Revenues by Product:

Net Product Revenues by Product

(In millions of U.S. dollars)

	Three Months Ended June 30,			Six Months Ended June 30,		
	2020	**2019**	**% Change**	**2020**	**2019**	**% Change**
	(unaudited)	(unaudited)		(unaudited)	(unaudited)	
Brineura	$ 25.8	$ 14.8	74 %	$ 49.8	$ 27.0	84 %
Firdapse [(1)]	—	5.5	(100) %	1.2	10.6	(89) %
Naglazyme	81.0	98.2	(18) %	195.3	185.1	6 %
PKU franchise	163.3	132.1	24 %	319.9	251.3	27 %
Vimizim	116.7	122.7	(5) %	253.9	248.5	2 %

Net Product Revenues Marketed by BioMarin	386.8	373.3		820.1	722.5	
Aldurazyme Net Product Revenues Marketed by Genzyme	32.3	5.8	457 %	88.0	51.1	72 %
Total Net Product Revenues	$ 419.1	$ 379.1		$ 908.1	$ 773.6	

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

The following table presents Net Product Revenues for the PKU Franchise by Product:

Net Product Revenues by Product for the PKU Franchise

(In millions of U.S. dollars)

(unaudited)

	Three Months Ended June 30,			Six Months Ended June 30,		
	2020	2019	% Change	2020	2019	% Change
	(unaudited)	(unaudited)		(unaudited)	(unaudited)	
Kuvan	$ 122.6	113.3	8 %	$ 244.6	220.2	11 %
Palynziq	40.7	18.8	116 %	75.3	31.1	142 %
Total PKU franchise	$ 163.3	$ 132.1	24 %	$ 319.9	$ 251.3	27 %

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 June 30,		Six Months Ended June 30,		Guidance Year Ending		
	2020	2019	2020	2019	December 31, 2020		
GAAP Net Income (Loss)	**$ (29.2)**	**$ (37.4)**	**$ 52.2**	**$ (93.9)**	**$ 720.0**	**—**	**$ 980.0**
Interest expense, net	3.7	1.0	5.4	1.4	3.0	—	2.0
Provision for (benefit from) income taxes	(13.1)	(4.5)	6.9	(0.9)	(721.8)	—	(915.8)
Depreciation expense	9.7	12.9	20.0	27.9	50.0	—	47.0
Amortization expense	15.5	13.4	31.2	20.9	63.0	—	61.0
Stock-based compensation expense	45.1	39.8	92.0	82.6	177.0	—	167.0
Contingent consideration expense	(0.6)	6.9	(0.6)	19.2	2.0	—	2.0
Gain on sale of nonfinancial assets	—	(15.0)	(59.5)	(15.0)	(59.5)	—	(59.5)

Licensed In-Process R&D [(1)]	26.3	—	26.3	—	26.3	—	26.3
Non-GAAP Income	$ 57.4	$ 17.1	$ 173.9	$ 42.2	$ 260.0	—	$ 310.0

(1) Represents the one time 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 June 30,

	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-Based Compensation, Contingent Consideration and Other Adjustments**	**Non-GAAP**
Cost of sales	**$ 97.9**	$ —	$ (4.9)	$ 93.0	**$ 77.4**	$ —	$ (3.7)	$ 73.7
Research and development	**182.1**	(4.6)	(42.3)	135.2	**185.6**	(7.3)	(14.9)	163.4
Selling, general and administrative	**175.4**	(5.1)	(24.2)	146.1	**160.8**	(5.6)	(21.2)	134.0
Intangible asset amortization and contingent consideration	**14.9**	(15.5)	0.6	—	**20.3**	(13.4)	(6.9)	—
Gain on sale of nonfinancial assets	**—**	—	—	—	**(15.0)**	—	15.0	—
Interest expense, net	**(3.7)**	3.7	—	—	**(1.0)**	1.0	—	—
Provision for (benefit from) income taxes	**(13.1)**	13.1	—	—	**(4.5)**	4.5	—	—
GAAP Net Income (Loss)/Non-GAAP Income	**$ (29.2)**	$ 15.8	$ 70.8	$ 57.4	**$ (37.4)**	$ 22.8	$ 31.7	$ 17.1

Six months ended June 30,

				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-Based Compensation, Contingent Consideration and Other Adjustments**	**Non-GAAP**
Cost of sales	**$ 209.3**	$ 0.0	$ (10.0)	$ 199.3	**$ 166.6**	$ 0.0	(8.5)	$ 158.1
Research and development	**324.4**	(9.4)	(56.0)	259.0	**369.2**	(16.7)	(28.8)	323.7
Selling, general and administrative	**362.7**	(10.6)	(52.3)	299.8	**322.9**	(11.2)	(45.3)	266.4
Intangible								

asset amortization and contingent consideration	**30.6**	(31.2)	0.6	—	**40.1**	(20.9)	(19.2)	—
Gain on sale of nonfinancial assets	**(59.5)**	—	59.5	—	**(15.0)**	—	15.0	—
Interest expense, net	**(5.4)**	5.4	—	—	**(1.4)**	1.4	—	—
Provision for (benefit from) income taxes	**6.9**	(6.9)	—	—	**(0.9)**	0.9	—	—
GAAP Net Loss/Non-GAAP Income	**52.2**	63.5	58.2	173.9	**(93.9)**	49.3	86.8	42.2

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