BioMarin Announces Second Quarter 2021 Financial Results and Corporate Updates

- Total Revenues Grew 17% in the Second Quarter 2021 Compared to Second Quarter 2020

- Raising Full-year 2021 Financial Guidance

- Marketing Authorization Application (MAA) for Valoctocogene Roxaparvovec, for the Treatment of Severe Hemophilia A, Validated by European Medicines Agency; CHMP Opinion Expected First Half 2022

- Positive CHMP Opinion Received in Europe for VOXZOGO™ for the Treatment of Children with Achondroplasia from Age 2 until Growth Plates Close; European Commission Decision expected in the Third Quarter 2021

- FDA Review of VOXZOGO Proceeding; PDUFA Target Action Date November 20, 2021

- BioMarin to Showcase Development Pipeline at R&D Day November 2021

SAN RAFAEL, Calif., July 28, 2021 /PRNewswire/ --

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

	Three Months Ended June 30,			Six Months Ended June 30,		
	2021	2020	% Change	2021	2020	% Change
Total Revenues	$ 501.7	$ 429.5	17 %	$ 987.7	$ 931.6	6 %
Net Product Revenues Marketed by BioMarin (1)	$ 458.6	$ 386.8	19 %	$ 876.3	$ 820.1	7 %
Vimizim Net Product Revenues	$ 171.7	$ 116.7	47 %	$ 329.9	$ 253.9	30 %
Naglazyme Net Product Revenues	$ 118.8	$ 81.0	47 %	$ 226.1	$ 195.3	16 %
Kuvan Net Product Revenues	$ 78.8	$ 122.6	(36) %	$ 149.6	$ 244.6	(39) %
Palynziq Net Product Revenues	$ 59.0	$ 40.7	45 %	$ 113.0	$ 75.3	50 %
Brineura Net Product Revenues	$ 30.3	$ 25.8	17 %	$ 57.7	$ 49.8	16 %
Aldurazyme Net Product Revenues	$ 28.1	$ 32.3	(13) %	$ 78.1	$ 88.0	(11) %
GAAP Net Income (Loss)	$ 12.9	$ (29.2)		$ 30.3	$ 52.2	
GAAP Net Income (Loss) per Share – Basic	$ 0.07	$ (0.16)		$ 0.17	$ 0.29	
GAAP Net Income (Loss) per Share – Diluted	$ 0.07	$ (0.16)		$ 0.16	$ 0.28	
Non-GAAP Income (2)	$ 97.8	$ 57.4		$ 202.1	$ 173.9	

	June 30, 2021	December 31, 2020
Cash, cash equivalents and investments	$ 1,473.6	$ 1,350.9

(1) Net Product Revenues Marketed by BioMarin is the sum of revenues from Vimizim, Kuvan, Naglazyme, Palynziq, Brineura for the three and six months ended June 30, 2021 and includes Firdapse for the six months ended June 30, 2020, 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 **10** 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 first quarter ended March 31, 2020; as a result of the transaction BioMarin no longer recognizes Net Product Revenues from Firdapse.

(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 **11** 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, 2021.

"We enjoyed a strong first half of 2021, including financial results and the achievement of our key regulatory goals," said Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin. "With the positive CHMP opinion for VOXZOGO for children, 2 years of age and older, our commercial team is eagerly preparing for potential European approval and launch later this summer. If approved, VOXZOGO would be the first pharmacological treatment option for children with achondroplasia. Following potential approval in Europe later this summer, we hope to have a positive outcome in the United States pending our November 20 PDUFA target action date. Upon potential global approvals of VOXZOGO for achondroplasia, we have a tremendous opportunity to address the interest from families seeking treatment for their children."

Mr. Bienaimé continued, "We were pleased to receive validation of our MAA in Europe for valoctocogene roxaparvovec for the treatment of severe hemophilia A, paving the way for a CHMP opinion in the first half of 2022. Our data presentations at the International Society on Thrombosis and Haemostasis in July provided further evidence of hemostatic efficacy following treatment with valoctocogene roxaparvovec, including significant reductions in bleeding events and Factor VIII infusions. We look forward to two year data results from our complete Phase 3 study with 134 participants in early 2022, and anticipate submitting a BLA in the second quarter of 2022, should data be supportive. More than ever, we believe that valoctocogene roxaparvovec gene therapy will be an important treatment option for people with severe hemophilia A. Given the superior bleed control seen at one year with valoctocogene roxaparvovec, and the burden of frequent, regular infusions with standard of care prophylaxis, we believe one infusion of valoctocogene roxaparvovec gene therapy has the potential to address the unmet need for bleed control, supported by our clinical results to date."

Financial Highlights:

- **Total Revenues** for the second quarter of 2021 increased to $501.7 million, compared to $429.5 million for the same period in 2020. The increase in Total Revenues was primarily attributed to the following:
    - Higher Vimizim and Naglazyme product revenues primarily driven by timing of orders from Europe and Middle East; and
    - Higher Palynziq product revenues primarily driven by a combination of revenue from more U.S. patients achieving maintenance dosing and new patients initiating therapy; partially offset by
    - Lower Kuvan product revenues primarily due to generic competition due to the loss of exclusivity in the U.S.
- **GAAP Net Income** increased to $12.9 million for the second quarter of 2021 compared to a GAAP Net Loss of $29.2 million for the same period in 2020. The increase was primarily due to profits from higher revenues and lower research and development (R&D) expense driven by lower in-licensing expenses.
- **Non-GAAP Net Income** for the second quarter of 2021 increased to $97.8 million compared to Non-GAAP Income of $57.4 million for the same period in 2020. The increase in Non-GAAP Income for the quarter, compared to the same period in 2020, was primarily attributed to higher gross profit partially offset by higher SG&A expense.

Commercial Portfolio (Naglazyme, Vimizim, Brineura, Palynziq, Kuvan and Aldurazyme)

- Naglazyme, Vimizim and Brineura maintained robust patient compliance in the quarter and continue to grow based on strong underlying demand.
    - The number of patients on commercial Naglazyme and Vimizim therapy increased by over 10% each year-over-year.
    - The number of commercial patients on commercial Brineura therapy increased by over 30% year-over-year.
- Order timing for both Naglazyme and Vimizim for the full year 2021 are expected to be more concentrated in the first half of the year.
- Palynziq top-line results for the full year 2021 are expected to increase approximately 40% as compared to full-year 2020 results based on the mid-point of full-year 2021 guidance.
- Palynziq growth in the European, Middle East and African regions (EMEA) has been impacted by ongoing challenges due to COVID-19 with Palynziq revenue from EMEA expected to increase when PKU clinics have more freedom to operate and start treating additional patients.
    - The number of U.S. patients on commercial Palynziq therapy increased approximately 30% year-over-year and is expected to continue to grow as U.S. PKU clinics are expected to re-open over the coming quarters.
- Loss of Kuvan U.S. market share to generics was consistent with expectations.

Late-stage Regulatory Portfolio (VOXZOGO and valoctocogene roxaparvovec)

- In Europe, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion of VOXZOGO for the treatment of children, ages 2 years and older. A final approval decision, typically consistent with the CHMP recommendation, is expected from the European Commission in the third quarter of 2021.
- The U.S. Food and Drug Administration (FDA) is reviewing the VOXZOGO New Drug Application (NDA) with the Prescription Drug User Fee Act (PDUFA) target action date of November 20, 2021.

- The European Medicines Agency (EMA) validated BioMarin's MAA for valoctocogene roxaparvovec: CHMP opinion anticipated in the second quarter of 2022.
- BioMarin is targeting a Biologics License Application (BLA) submission for valoctocogene roxaparvovec in the second quarter of 2022 assuming favorable results from the two-year follow-up safety and efficacy data from the GENEr8-1 study, followed by an expected 6-month review procedure by the FDA.

Earlier-stage Development Portfolio (BMN 307, BMN 255, BMN 331, DiNA-001, Allen Institute Collaboration)

- BMN 307: Dose escalation in PHEarless, the Phase 1/2 study of BMN 307 continues based on encouraging Phe lowering and safety profile observed in study participants who were treated with the lowest dose.
- BMN 255 for a subset of chronic renal disease: BMN 255 is a small molecule for the treatment of a subset of chronic renal disease. The Investigational New Drug application (IND) for BMN 255 is active and the Company is dosing subjects.
- BMN 331 gene therapy product candidate for Hereditary Angioedema (HAE): BMN 331 is BioMarin's third gene therapy candidate. 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. The IND for BMN 331 was recently cleared by FDA and is active.
- BMN 351 for Duchenne Muscular Dystrophy (DMD): IND-enabling studies continue with BMN 351, an antisense oligonucleotide therapy that has demonstrated dystrophin expression levels of 30-50% of wild-type levels in the quadriceps in a DMD mouse model treated at 18.7 mg/kg/week for 13 weeks (measured 2 weeks following last administration). If results from the ongoing pre-clinical studies are supportive, BioMarin anticipates filing an IND for BMN 351 in the first half of 2022.
- 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.
- On April 28, 2021, BioMarin and the Allen Institute announced a collaboration to create new gene therapies aimed at rare genetic diseases of the central nervous system (CNS). The goal is to combine the Allen Institute's leadership in large-scale genomic science in the CNS therapeutic area with BioMarin's expertise in developing transformational gene therapies.

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

Item	Provided April 29, 2021*			Revised July 28, 2021*		
Total Revenues	$1,750	to	$1,850	$1,790	to	$1,880
Vimizim Net Product Revenues	$570	to	$610	$580	to	$620
Kuvan Net Product Revenues	$250	to	$290	$260	to	$290
Naglazyme Net Product Revenues	$365	to	$395	$375	to	$405
Palynziq Net Product Revenues	$210	to	$250	$220	to	$260
Brineura Net Product Revenues	$120	to	$140	Unchanged		
Cost of Sales (% of Total Revenues)	23%	to	25%	Unchanged		
Research and Development Expense	$645	to	$695	$645	to	$685
Selling, General and Administrative Expense	$725	to	$775	$735	to	$775
GAAP Net Loss	($130)	to	($80)	($110)	to	($60)
Non-GAAP Income [(1)]	$170	to	$220	$190	to	$240

*2021 Guidance takes into consideration ongoing expected impact from the COVID-19 pandemic assuming consistent trends experienced during 2020 and the first two quarters of 2021.

(1) All Financial Guidance items are calculated based on U.S. GAAP with the exception of Non-GAAP Income. 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.

BioMarin will host a conference call and webcast to discuss second quarter and full-year 2021 financial results today, Wednesday, July 28, 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: 8363378	Conference ID: 8363378

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; cash flows from operating activities; the timing of orders for commercial products; expectations regarding the ongoing impact of the COVID-19 pandemic; 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 plans to re-submit a BLA for valoctocogene roxaparvovec to the FDA with two-year follow-up results from all the subjects from the Phase 3 GENEr8-1 study in the second quarter of 2022, (ii) BioMarin's anticipated IND submission for BMN 351 in the first half of 2022, and (iii) BioMarin and Allen Institute collaborating to create new gene therapies; the potential approval and commercialization of BioMarin's product candidates, including VOXZOGO for the treatment of achondroplasia and valoctocogene roxaparvovec for the treatment of severe hemophilia A; the timing of such approval decisions, including (i) the expectation of a CHMP opinion in the second quarter of 2022 for valoctocogene roxaparvovec, (ii) the expectation of a European Commission Decision in the third quarter of 2021 following the receipt of the positive CHMP opinion for VOXZOGO and (iii) the extended PDUFA target action date with respect to VOXZOGO of November 20, 2021; and the expected benefits and availability of BioMarin's product candidates, including that VOXZOGO would be the first potential pharmacological therapeutic option for children with achondroplasia; and potential growth opportunities and trends, including (i) that BioMarin would have a tremendous opportunity to address the interest from families seeking treatment for their children upon potential global approval of VOXZOGO, (ii) that BioMarin believes that one infusion of valoctocogene roxaparvovec gene therapy has the potential to address the unmet need for bleed control and (iii) growth expectations regarding Palynziq.

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 March 31, 2021 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, 2021 and December 31, 2020

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

	June 30, 2021	December 31, 2020(1)

ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 641,533	$ 649,158
Short-term investments	481,864	416,228
Accounts receivable, net	424,419	448,351
Inventory	710,975	698,548
Other current assets	99,046	129,934
Total current assets	2,357,837	2,342,219
Noncurrent assets:		
Long-term investments	350,237	285,473
Property, plant and equipment, net	1,026,579	1,032,471
Intangible assets, net	394,298	417,271
Goodwill	196,199	196,199
Deferred tax assets	1,431,683	1,432,150
Other assets	141,663	142,237
Total assets	$ 5,898,496	$ 5,848,020
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 411,132	$ 492,548
Short-term contingent consideration	32,212	—
Total current liabilities	443,344	492,548
Noncurrent liabilities:		
Long-term convertible debt, net	1,077,110	1,075,145
Long-term contingent consideration	30,760	60,130
Other long-term liabilities	105,711	114,195
Total liabilities	$ 1,656,925	$ 1,742,018
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 183,321,820 and 181,740,999 shares issued and outstanding, respectively.	183	182
Additional paid-in capital	5,083,831	4,993,407
Company common stock held by Nonqualified Deferred Compensation Plan	(10,207)	(9,839)
Accumulated other comprehensive loss	(939)	(16,139)
Accumulated deficit	(831,297)	(861,609)
Total stockholders' equity	4,241,571	4,106,002
Total liabilities and stockholders' equity	$ 5,898,496	$ 5,848,020

(1) December 31, 2020 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, 2020, filed with the U.S. Securities and Exchange Commission (SEC) on February 26, 2021.

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

Three and Six Months Ended June 30, 2021 and 2020

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

	Three Months Ended June 30,	Six Months Ended June 30,

	2021 (unaudited)	2020 (unaudited)	2021 (unaudited)	2020 (unaudited)
REVENUES:				
Net product revenues	$ 486,670	$ 419,032	$ 954,439	$ 908,075
Royalty and other revenues	15,023	10,453	33,284	23,479
Total net revenues	501,693	429,485	987,723	931,554
OPERATING EXPENSES:				
Cost of sales	127,062	97,967	247,228	209,341
Research and development	161,107	182,139	309,832	324,396
Selling, general and administrative	184,161	175,412	358,479	362,707
Intangible asset amortization and contingent consideration	17,691	14,912	35,426	30,589
Gain on sale of nonfinancial assets	—	—	—	(59,495)
Total operating expenses	490,021	470,430	950,965	867,538
INCOME (LOSS) FROM OPERATIONS	11,672	(40,945)	36,758	64,016
Equity in the loss of BioMarin/Genzyme LLC	(175)	(79)	(1,526)	(156)
Interest income	4,471	4,291	6,910	9,535
Interest expense	(3,817)	(8,048)	(7,621)	(14,963)
Other income, net	2,005	2,508	2,863	647
INCOME (LOSS) BEFORE INCOME TAXES	14,156	(42,273)	37,384	59,079
Provision for (benefit from) income taxes	1,215	(13,090)	7,072	6,881
NET INCOME (LOSS)	12,941	(29,183)	$ 30,312	$ 52,198
NET INCOME (LOSS) PER SHARE, BASIC	$ 0.07	$ (0.16)	$ 0.17	$ 0.29
NET INCOME (LOSS) PER SHARE, DILUTED	$ 0.07	$ (0.16)	$ 0.16	$ 0.28
Weighted average common shares outstanding, basic	182,844	180,729	182,311	180,314
Weighted average common shares outstanding, diluted	185,427	180,729	185,089	184,344

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

Six Months Ended June 30, 2021 and 2020

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

	Six Months Ended June 30,	
	2021	**2020**
	(unaudited)	(unaudited)
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net income	$ 30,312	$ 52,198
Adjustments to reconcile net income to net cash used in operating activities:		
Depreciation and amortization	55,214	51,180
Non-cash interest expense	2,082	9,534
Amortization of premium on investments	1,878	38
Stock-based compensation	104,346	91,956
Gain on sale of nonfinancial assets	—	(59,495)
Deferred income taxes	1,204	(5,864)
Unrealized foreign exchange loss (gain)	(1,004)	2,201
Non-cash changes in the fair value of contingent consideration	4,488	(584)

Other	(376)	(650)
Changes in operating assets and liabilities:		
Accounts receivable, net	17,420	(8,028)
Inventory	6,379	(46,298)
Other current assets	34,331	20,441
Other assets	321	(5,599)
Accounts payable and accrued liabilities	(60,884)	(94,425)
Other long-term liabilities	585	5,938
Net cash provided by operating activities	196,296	12,543
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property, plant and equipment	(48,106)	(66,716)
Maturities and sales of investments	348,941	170,111
Purchases of available-for-sale securities	(484,572)	(321,684)
Proceeds from sale of nonfinancial assets	—	67,159
Purchase of intangible assets	(6,400)	(10,786)
Investment in convertible note	—	(8,709)
Other	(908)	(722)
Net cash used in investing activities	(191,045)	(171,347)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from exercises of awards under equity incentive plans	27,640	46,927
Taxes paid related to net share settlement of equity awards	(38,731)	(36,645)
Proceeds from convertible senior subordinated note offering, net	—	585,782
Repurchase of common stock	—	(50,000)
Principal repayments of financing leases	(1,941)	(2,047)
Other	(381)	—
Net cash provided by (used in) financing activities	(13,413)	544,017
Effect of exchange rate changes on cash	537	(3,759)
NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	$ (7,625)	$ 381,454
Cash and cash equivalents:		
Beginning of period	$ 649,158	$ 437,446
End of period	$ 641,533	$ 818,900

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,		
	2021	**2020**	**% Change**	**2021**	**2020**	**% Change**
	(unaudited)	(unaudited)		(unaudited)	(unaudited)	
Vimizim	$ 171.7	$ 116.7	47 %	$ 329.9	$ 253.9	30 %
PKU franchise	137.8	163.3	(16) %	262.6	319.9	(18) %
Naglazyme	118.8	81.0	47 %	226.1	195.3	16 %
Brineura	30.3	25.8	17 %	57.7	49.8	16 %
Firdapse [1]	—	—	— %	—	1.2	(100) %

Net Product Revenues Marketed by BioMarin	$ 458.6	$ 386.8		$ 876.3	$ 820.1	
Aldurazyme Net Product Revenues Marketed by Genzyme	28.1	32.3	(13) %	78.1	88.0	(11) %
Total Net Product Revenues	$ 486.7	$ 419.1		$ 954.4	$ 908.1	

(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 six months ending June 30, 2020; and as a result of the transaction BioMarin no longer generates Net Product Revenues from Firdapse.

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)

	Three Months Ended June 30,			Six Months Ended June 30,		
	2021	**2020**	**% Change**	**2021**	**2020**	**% Change**
	(unaudited)	(unaudited)		(unaudited)	(unaudited)	
Kuvan	$ 78.8	$ 122.6	(36) %	$ 149.6	$ 244.6	(39) %
Palynziq	59.0	40.7	45 %	113.0	75.3	50 %
Total PKU franchise	$ 137.8	$ 163.3	(16) %	$ 262.6	$ 319.9	(18) %

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 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
	2021	2020	2021	2020	December 31, 2021
GAAP Net Income (Loss)	**$ 12.9**	**$ (29.2)**	**$ 30.3**	**$ 52.2**	**$ (110.0) — $ (60.0)**
Interest expense (income), net	(0.7)	3.7	0.7	5.4	11.0 — 11.0
Provision for (benefit from) income taxes	1.2	(13.1)	7.1	6.9	(16.0) — (16.0)
Depreciation expense	11.8	9.7	24.3	20.0	48.0 — 48.0
Amortization expense	15.5	15.5	30.9	31.2	62.0 — 62.0
Stock-based compensation expense	54.9	45.1	104.3	92.0	186.0 — 186.0
Contingent consideration expense (benefit)	2.2	(0.6)	4.5	(0.6)	9.0 — 9.0
Gain on sale of nonfinancial assets	—	—	—	(59.5)	— — —
Licensed In-Process R&D [(1)]	—	26.3	—	26.3	— — —
Non-GAAP Income	$ 97.8	$ 57.4	$ 202.1	$ 173.9	$ 190.0 — $ 240.0

(1) 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 June 30,							
	2021				2020			
		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	C
Cost of sales	**$ 127.1**	$ —	$ (6.1)	$ 121.0	**$ 97.9**	$ —	$ (4.9)	$
Research and development	**161.1**	(7.1)	(20.8)	133.2	**182.1**	(4.6)	(42.3)	1
Selling, general and administrative	**184.2**	(4.7)	(28.0)	151.5	**175.4**	(5.1)	(24.2)	1
Intangible asset amortization and contingent consideration	**17.7**	(15.5)	(2.2)	—	**14.9**	(15.5)	0.6	
Interest income								

(expense), net	**0.7**	(0.7)	—	—	**(3.7)**	3.7	—	
Provision for (benefit from) income taxes	**1.2**	(1.2)	—	—	**(13.1)**	13.1	—	
GAAP Net Income (Loss) / Non-GAAP Income	**$ 12.9**	$ 27.8	$ 57.1	$ 97.8	**$ (29.2)**	$ 15.8	$ 70.8	$

	Six months ended June 30,							
	2021				**2020**			
		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**	
Cost of sales	**$ 247.2**	$ —	$ (12.6)	$ 234.6	**$ 209.3**	$ —	$ (10.0)	$
Research and development	**309.8**	(14.6)	(38.3)	256.9	**324.4**	(9.4)	(56.0)	
Selling, general and administrative	**358.5**	(9.7)	(53.4)	295.4	**362.7**	(10.6)	(52.3)	
Intangible asset amortization and contingent consideration	**35.4**	(30.9)	(4.5)	—	**30.6**	(31.2)	0.6	
Gain on sale of nonfinancial assets	**0.0**	—	—	—	**(59.5)**	—	59.5	
Interest expense, net	**(0.7)**	0.7	—	—	**(5.4)**	5.4	—	
Provision for income taxes	**7.1**	(7.1)	—	—	**6.9**	(6.9)	—	
GAAP Net Income / Non-GAAP Income	**$ 30.3**	$ 63.0	$ 108.8	$ 202.1	**$ 52.2**	$ 63.5	$ 58.2	$

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https://investors.biomarin.com/2021-07-28-BioMarin-Announces-Second-Quarter-2021-Financial-Results-and-Corporate-Updates