BioMarin Announces Third Quarter 2021 Financial Results and Corporate Updates

- Strong Commercial Launch of VOXZOGO™ for the Treatment of Children with Achondroplasia Ages 2 and Older Underway across Europe; U.S. Review Proceeding, Target Action Date Tracking to November 20, 2021

- European Opinion on the Marketing Authorization Application (MAA) for Valoctocogene Roxaparvovec, for the Treatment of Severe Hemophilia A, Expected First Half 2022; U.S. Resubmission of Biologics License Application (BLA) in U.S. on Track for Second Quarter 2022

- Improving Full-year 2021 Total Revenue and Bottom-line Financial Guidance

- Multiple Early-stage Programs to be Shared at Upcoming BioMarin R&D Day on November 30, 2021

SAN RAFAEL, Calif., Oct. 27, 2021 /PRNewswire/ --

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2021	2020	% Change	2021	2020	% Change
Total Revenues	$ 408.7	$ 476.8	(14) %	$ 1,396.5	$ 1,408.3	(1) %
Net Product Revenues Marketed by BioMarin (1)	$ 369.5	$ 419.8	(12) %	$ 1,245.8	$ 1,239.9	— %
Vimizim Net Product Revenues	$ 136.9	$ 147.9	(7) %	$ 466.8	$ 401.8	16 %
Naglazyme Net Product Revenues	$ 71.2	$ 76.3	(7) %	$ 297.3	$ 271.6	9 %
Kuvan Net Product Revenues	$ 67.7	$ 124.1	(45) %	$ 217.3	$ 368.7	(41) %
Palynziq Net Product Revenues	$ 60.7	$ 46.1	32 %	$ 173.7	$ 121.4	43 %
Brineura Net Product Revenues	$ 32.9	$ 25.4	30 %	$ 90.6	$ 75.2	20 %
Voxzogo Net Product Revenues	$ 0.1	$ —	n/a	$ 0.1	$ —	n/a
Aldurazyme Net Product Revenues	$ 24.4	$ 40.9	(40) %	$ 102.5	$ 128.9	(20) %
GAAP Net Income (Loss)	$ (36.5)	$ 784.8		$ (6.2)	$ 837.0	
GAAP Net Income (Loss) per Share – Basic	$ (0.20)	$ 4.33		$ (0.03)	$ 4.63	
GAAP Net Income (Loss) per Share – Diluted	$ (0.20)	$ 4.01		$ (0.03)	$ 4.39	
Non-GAAP Income (2)	$ 33.5	$ 98.7		$ 235.7	$ 272.6	

	September 30, 2021	December 31, 2020
Cash, cash equivalents and investments	$ 1,546.1	$ 1,350.9

(1) Net Product Revenues Marketed by BioMarin is the sum of revenues from Vimizim, Naglazyme, Kuvan, Palynziq, Brineura and Voxzogo (formerly known as vosoritide) for the three and nine months ended September 30, 2021 and includes Firdapse for the nine months ended September 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 nine months ended September 30, 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 third quarter ended September 30, 2021.

"As we begin the last quarter of 2021, we are very pleased to be improving both top and bottom-line financial guidance for the full-year," said Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin. "Uneven ordering patterns are a consistent element of our dynamic global commercial business, making full-year results the key indicator of product demand. The essential nature of our medicines to the people who rely on them, gives us confidence in the growth prospects ahead. We plan to finish 2021 strong and expect meaningful growth in revenues in 2022 leading to anticipated GAAP profitability next year. We look forward to providing full guidance for 2022 early next year, especially given early demand signals for Voxzogo, for the treatment of achondroplasia, in Europe, and the potential for Voxzogo to be approved in the U.S. next month."

Mr. Bienaimé continued, "Foundational milestones are tracking to plan, starting with the European approval and launch of Voxzogo in the quarter. The level of interest in Voxzogo following approval has exceeded our expectations. If Voxzogo is approved in the U.S. next month, we expect the global Voxzogo launch to contribute meaningfully to our commercial business. Our next significant read-out, the two-year data from the 134-participant Phase 3 study with valoctocogene roxaparvovec gene therapy for the treatment of severe hemophilia A, will be shared in early 2022. We anticipate resubmitting the U.S. application in the second quarter of 2022, and we continue to anticipate the European opinion in the second quarter of 2022, should data be supportive. The cascade of these potential new product approvals, including global approvals of valoctocogene roxaparvovec and U.S. approval of Voxzogo, sets us up for significant potential growth beginning in 2022."

Financial Highlights:

- **Total Revenues** for the third quarter of 2021 decreased to $408.7 million, compared to $476.8 million for the same period in 2020. The decrease in Total Revenues was primarily attributed to the following:
    - Lower Kuvan product revenues primarily due to generic competition due to the loss of exclusivity in the U.S., which was consistent with expectations.
    - Lower Aldurazyme product revenues due to timing of product fulfillment to Genzyme. BioMarin Aldurazyme revenues are driven by the timing of when the product is released and control is transferred to Genzyme to be marketed. Revenues for the third quarter of 2021 were comparatively lower than 2020 due to such timing. Based on data provided to us by Genzyme, patients receiving commercial Aldurazyme increased by approximately 10%, year-over-year. Aldurazyme 2021 revenues are expected to grow in the fourth quarter of 2021 compared to the fourth quarter of 2020.
    - Lower Vimizim and Naglazyme product revenues primarily driven by timing of orders from Europe and the Middle East. High patient compliance rates during the quarter and year-over-year patient growth of approximately 10% for both Naglazyme and Vimizim reflected strong underlying demand.

      These factors were partially offset by the following:

    - Higher Palynziq product revenues primarily due to a combination of revenues from more patients in the U.S. achieving maintenance dosing and new patients initiating therapy, which increased approximately 20% year-over-year. We expect the number of U.S. Palynziq patients to grow as U.S. PKU clinics re-open over the coming months. The commercial launch of Palynziq in BioMarin's European, Middle East and African (EMEA) region continues to progress through individual country level pricing and reimbursement negotiations.
    - Higher Brineura product revenues due to new patients initiating therapy driven by growth in EMEA and North America. Strong patient compliance during the quarter and year-over-year patient growth of approximately 20% reflected strong underlying demand.
- Total Net Product Revenues Marketed by BioMarin, excluding Kuvan, increased in the third quarter 2021 compared to the third quarter 2020.
- **GAAP Net Loss** increased to $36.5 million for the third quarter of 2021 compared to GAAP Net Income of $784.8 million for the same period in 2020. GAAP Net Income in the third quarter of 2020 was primarily related to the $835.1 million benefit from income taxes related to the intra-entity transfer of certain intellectual property rights to an Irish subsidiary, and there was no similar transaction in the third quarter of 2021.
- **Non-GAAP Net Income** for the third quarter of 2021 decreased to $33.5 million compared to Non-GAAP Income of $98.7 million for the same period in 2020. The decrease in Non-GAAP Income for the quarter, compared to the same period in 2020, was primarily attributable to lower net product revenues.

Late-stage Regulatory Portfolio (Voxzogo and valoctocogene roxaparvovec)

- During the quarter, the European Commission approved Voxzogo for the treatment of children, ages 2 years and older. The commercial launch of Voxzogo is underway, with revenues for the remainder of 2021 expected from France, Germany and select early-access countries. Marketing authorization reviews are in process in Japan, Brazil and Australia, with potential approvals in those countries in 2022.
- The FDA is reviewing the Voxzogo New Drug Application (NDA) with the PDUFA target action date of November 20, 2021.
- The European Medicines Agency (EMA) validated BioMarin's MAA for valoctocogene roxaparvovec resulting in an anticipated CHMP

opinion in the first half of 2022, assuming favorable results from the two-year follow-up safety and efficacy data from the GENEr8-1 study.

- BioMarin is targeting a BLA resubmission for valoctocogene roxaparvovec in the second quarter of 2022, also 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, BMN 351, DiNA-001, Allen Institute Collaboration, Deep Genomics)

- Details for BioMarin's virtual R&D Day on November 30, 2021 will be made available over the coming weeks. The Company plans to share an in-depth review of multiple early-stage pipeline programs showcasing next potential IND candidates.
- BMN 307 gene therapy product candidate for PKU: The FDA placed a clinical hold on PHEarless, the Phase 1/2 study evaluating BMN 307, an investigational AAV5-phenylalanine hydroxylase (PAH) gene therapy, in adults with phenylketonuria (PKU). The hold was based on pre-clinical study findings from a model designed to understand the durability of BMN 307 activity in mice bearing two germline mutations, one rendering the mice immunodeficient. The clinical significance of these findings is being evaluated to assure safe and appropriate use of BMN 307. To date, findings appear specific to mice and have no known translatability to humans or other gene therapy vectors. BioMarin is working with health authorities to address the clinical hold and resume study investigations, as appropriate.
- BMN 255 for a subset of chronic renal disease: The Investigational New Drug application (IND) for BMN 255 is active and the Company is dosing subjects. Kidney damage can progress to end stage renal disease, and the availability of a potent, orally bioavailable, small molecule like BMN 255, may be able to significantly reduce disease and treatment burden in certain people with chronic renal disease.
- BMN 331 gene therapy product candidate for Hereditary Angioedema (HAE): BioMarin is leveraging its broad expertise in gene therapy to improve efficiencies in the development process of BMN 331. The IND for BMN 331 has been allowed by FDA and the first patient is expected to be enrolled around the end of 2021. Patients with HAE may experience benefit from the constitutive expression and stable C1-INH protein levels potentially provided by gene therapy, thus reducing the frequency and severity of attacks and reducing the burden associated with current standard of care.
- BMN 351 for Duchenne Muscular Dystrophy (DMD): IND-enabling studies continue with BMN 351, an antisense oligonucleotide therapy for individuals with 51-skip-amenable DMD. BMN 351 was developed using familiar chemistry and superior biology, by targeting a novel, upstream, splice enhancer site demonstrating improved binding affinity and metabolic stability in preclinical models. Preclinical data suggest that restored expression of near-full-length dystrophin protein at previously achieved levels of up to 40% will convert phenotypes from rapid loss to durable preservation of strength and ambulation. 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. Mutations in MYBPC3 are the most common cause of inherited HCM. Early investigations suggest that gene therapy-mediated gene transfer can lead to widespread expression of the gene product, cardiac myosin-binding protein C (MyBP-C), in cardiac tissue, which can normalize relaxation kinetics and potentially ameliorate the disease phenotype in individuals suffering from cardiomyopathy.
- BioMarin and the Allen Institute are collaborating 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 therapies for complex conditions, such as Brineura for CLN2. The phenotypes associated with some of these conditions have the potential to demonstrate profound recoverability and transformational outcomes, particularly when treated early. This partnership expands and fortifies BioMarin's capabilities and expertise in the CNS therapeutic area.
- Deep Genomics and BioMarin recently entered into a collaborative agreement to develop oligonucleotide drug candidates in four rare disease indications. Through this collaboration, BioMarin will advance lead compounds identified and validated using Deep Genomics' artificial intelligence (AI) drug discovery platform into preclinical and clinical development. As part of this collaboration, BioMarin receives an exclusive option to each of the four disease programs for development and commercialization. By using a combination of Deep Genomics' advanced analytics and BioMarin's expertise in rare disease drug development, the collaboration could efficiently unlock a path to identify molecular disease targets for novel therapeutics.

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

Item	Provided July 28, 2021*			Revised October 27, 2021*		
Total Revenues	$1,790	to	$1,880	$1,820	to	$1,880
Vimizim Net Product Revenues	$580	to	$620	$595	to	$620
Naglazyme Net Product Revenues	$375	to	$405	Unchanged		
Palynziq Net Product Revenues	$220	to	$260	$230	to	$260
Kuvan Net Product Revenues	$260	to	$290	$265	to	$290
Brineura Net Product Revenues	$120	to	$140	Unchanged		

Cost of Sales (% of Total Revenues)	23%	to	25%	Unchanged		
Research and Development Expense	$645	to	$685	$635	to	$675
Selling, General and Administrative Expense	$735	to	$775	Unchanged		
GAAP Net Loss	($110)	to	($60)	($85)	to	($45)
Non-GAAP Income [(1)]	$190	to	$240	$215	to	$255

*2021 Guidance takes into consideration ongoing expected impact from the COVID-19 pandemic assuming consistent trends experienced during 2020 and the nine months ended September 30, 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 11 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 third quarter and year to date 2021 financial results today, Wednesday, October 27, 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: 2382767	Conference ID: 2382767

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, including our expected read-out of two-year data from our Phase 3 study of valoctocogene roxaparvovec in early 2022; 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's collaborations with DiNAQOR, Allen Institute and Deep Genomics to create 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 potential approval of Voxzogo in the U.S. next month and in Japan, Brazil, and Australia in 2022, (ii) the expectation of a CHMP opinion in the first half of 2022 for valoctocogene roxaparvovec, 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; and potential growth opportunities and trends, including that BioMarin expects (i) meaningful growth in revenues in 2022 leading to anticipated GAAP profitability next year, (ii) Voxzogo to contribute meaningfully to its commercial business if approved in the U.S. next month, (iii) Aldurazyme revenues to grow in the fourth quarter of 2021 compared to the fourth quarter of 2020, and (iv) the number of U.S. Palynziq patients to grow as U.S. PKU clinics re-open.

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 June 30, 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.

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BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

September 30, 2021 and December 31, 2020

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

	September 30, 2021	December 31, 2020(1)
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 617,143	$ 649,158
Short-term investments	462,333	416,228
Accounts receivable, net	374,937	448,351
Inventory	749,406	698,548
Other current assets	107,751	129,934
Total current assets	2,311,570	2,342,219
Noncurrent assets:		
Long-term investments	466,618	285,473
Property, plant and equipment, net	1,024,787	1,032,471
Intangible assets, net	388,487	417,271
Goodwill	196,199	196,199
Deferred tax assets	1,445,109	1,432,150
Other assets	144,705	142,237
Total assets	$ 5,977,475	$ 5,848,020
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 466,711	$ 492,548
Short-term contingent consideration	48,187	—
Total current liabilities	514,898	492,548
Noncurrent liabilities:		
Long-term convertible debt, net	1,078,093	1,075,145
Long-term contingent consideration	15,204	60,130
Other long-term liabilities	103,131	114,195
Total liabilities	$ 1,711,326	$ 1,742,018
Stockholders' equity:		

Common stock, $0.001 par value: 500,000,000 shares authorized; 183,567,424 and 181,740,999 shares issued and outstanding, respectively.	184	182
Additional paid-in capital	5,133,742	4,993,407
Company common stock held by Nonqualified Deferred Compensation Plan	(10,225)	(9,839)
Accumulated other comprehensive income (loss)	10,239	(16,139)
Accumulated deficit	(867,791)	(861,609)
Total stockholders' equity	4,266,149	4,106,002
Total liabilities and stockholders' equity	$ 5,977,475	$ 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 Nine Months Ended September 30, 2021 and 2020

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	**2020**	**2021**	**2020**
	(unaudited)	(unaudited)	(unaudited)	(unaudited)
REVENUES:				
Net product revenues	$ 393,840	$ 460,741	$ 1,348,279	$ 1,368,816
Royalty and other revenues	14,902	16,043	48,186	39,522
Total revenues	408,742	476,784	1,396,465	1,408,338
OPERATING EXPENSES:				
Cost of sales	103,537	188,793	350,765	398,134
Research and development	157,869	147,053	467,701	471,449
Selling, general and administrative	183,333	179,450	541,812	542,157
Intangible asset amortization and contingent consideration	17,222	17,429	52,648	48,018
Gain on sale of nonfinancial assets	—	—	—	(59,495)
Total operating expenses	461,961	532,725	1,412,926	1,400,263
INCOME (LOSS) FROM OPERATIONS	(53,219)	(55,941)	(16,461)	8,075
Equity in the income (loss) of BioMarin/Genzyme LLC	177	(921)	(1,349)	(1,077)
Interest income	1,827	4,004	8,737	13,539
Interest expense	(3,870)	(9,597)	(11,491)	(24,560)
Other income, net	8,925	1,239	11,788	1,886
INCOME (LOSS) BEFORE INCOME TAXES	(46,160)	(61,216)	(8,776)	(2,137)
Benefit from income taxes	(9,666)	(846,019)	(2,594)	(839,138)
NET INCOME (LOSS)	(36,494)	784,803	$ (6,182)	$ 837,001
NET INCOME (LOSS) PER SHARE, BASIC	$ (0.20)	$ 4.33	$ (0.03)	$ 4.63
NET INCOME (LOSS) PER SHARE, DILUTED	$ (0.20)	$ 4.01	$ (0.03)	$ 4.39
Weighted average common shares outstanding, basic	183,214	181,142	182,616	180,592
Weighted average common shares outstanding, diluted	183,214	197,674	182,616	194,959

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

Nine Months Ended September 30, 2021 and 2020

(In thousands of U.S. dollars)

	Nine Months Ended September 30,	
	2021	**2020**
	(unaudited)	(unaudited)
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net income (loss)	$ (6,182)	$ 837,001
Adjustments to reconcile net income (loss) to net cash used in operating activities:		
Depreciation and amortization	82,053	77,814
Non-cash interest expense	3,114	14,766
Amortization of premium on investments	3,279	175
Stock-based compensation	153,372	142,125
Gain on sale of nonfinancial assets	—	(59,495)
Inventory reserves, net of stock-based compensation	—	75,609
Deferred income taxes	(12,020)	(854,199)
Unrealized foreign exchange (gain) loss	(1,347)	9,082
Non-cash changes in the fair value of contingent consideration	6,254	1,352
Other	(1,317)	388
Changes in operating assets and liabilities:		
Accounts receivable, net	65,513	(32,915)
Inventory	(19,125)	(73,310)
Other current assets	27,029	32,848
Other assets	(407)	(5,543)
Accounts payable and accrued liabilities	(7,129)	(76,174)
Other long-term liabilities	269	9,225
Net cash provided by operating activities	293,356	98,749
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property, plant and equipment	(66,840)	(83,286)
Maturities and sales of investments	502,112	345,224
Purchases of available-for-sale securities	(737,144)	(369,942)
Proceeds from sale of nonfinancial assets	—	67,159
Purchase of intangible assets	(8,026)	(14,369)
Investment in convertible note	—	(8,709)
Other	(994)	(725)
Net cash used in investing activities	(310,892)	(64,648)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from exercises of awards under equity incentive plans	32,877	60,268
Taxes paid related to net share settlement of equity awards	(44,428)	(42,667)
Repurchase of common stock	—	(50,000)
Proceeds from convertible senior subordinated note offering, net	—	585,752
Principal repayments of financing leases	(2,492)	(6,080)
Other	(401)	—
Net cash provided by (used in) financing activities	(14,444)	547,273
Effect of exchange rate changes on cash	(35)	(3,145)
NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	$ (32,015)	$ 578,229
Cash and cash equivalents:		

Beginning of period	$ 649,158	$ 437,446
End of period	$ 617,143	$ 1,015,675

The following table presents Net Product Revenues by Product:

Net Product Revenues by Product

(In millions of U.S. dollars)

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2021	**2020**	**% Change**	**2021**	**2020**	**% Change**
	(unaudited)	(unaudited)		(unaudited)	(unaudited)	
Vimizim	$ 136.9	$ 147.9	(7) %	$ 466.8	$ 401.8	16 %
Naglazyme	71.2	76.3	(7) %	297.3	271.6	9 %
Kuvan	67.7	124.1	(45) %	217.3	368.7	(41) %
Palynziq	60.7	46.1	32 %	173.7	121.4	43 %
Brineura	32.9	25.4	30 %	90.6	75.2	20 %
Voxzogo	0.1	—	n/a	0.1	—	n/a
Firdapse [(1)]	—	—	— %	—	1.2	(100) %
Net Product Revenues Marketed by BioMarin	$ 369.5	$ 419.8		$ 1,245.8	$ 1,239.9	
Aldurazyme Net Product Revenues Marketed by Genzyme	24.4	40.9	(40) %	102.5	128.9	(20) %
Total Net Product Revenues	$ 393.9	$ 460.7		$ 1,348.3	$ 1,368.8	

(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 nine months ended September 30, 2020; and as a result of the transaction BioMarin no longer generates Net Product Revenues from Firdapse.

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 September 30,		Nine Months Ended September 30,		Guidance Year Ending		
	2021	**2020**	**2021**	**2020**	**December 31, 2021**		
GAAP Net Income (Loss)	**$ (36.5)**	**$ 784.8**	**$ (6.2)**	**$ 837.0**	**$ (85.0)**	**— $ (45.0)**	
Interest expense, net	2.0	5.6	2.8	11.0	11.0	—	11.0
Benefit from income taxes	(9.7)	(846.0)	(2.6)	(839.1)	(16.0)	—	(16.0)
Depreciation expense	11.4	11.1	35.7	31.1	48.0	—	48.0
Amortization expense	15.4	15.5	46.3	46.7	62.0	—	62.0
Stock-based compensation expense	49.1	50.2	153.4	142.2	186.0	—	186.0
Contingent consideration expense	1.8	1.9	6.3	1.3	9.0	—	9.0
Provision for inventory reserve, net [1]	—	75.6	—	75.6	—	—	—
Gain on sale of nonfinancial assets	—	—	—	(59.5)	—	—	—
Licensed In-Process R&D [2]	—	—	—	26.3	—	—	—
Non-GAAP Income	$ 33.5	$ 98.7	$ 235.7	$ 272.6	$ 215.0	— $ 255.0	

(1) Represents a $81.2 million charge related to pre-launch valoctocogene roxaparvovec inventory, net of stock-based compensation, as a result of the unexpected delays in anticipated regulatory approvals.

(2) Represents the upfront license fee paid to a third party and recognized as R&D expense in the second quarter of 2020.

The following reconciliation of the GAAP reported to the Non-GAAP information provides the details of the effects of the Non-GAAP adjustments on certain components of the Company's operating results for each of the periods presented.

Reconciliation of Certain GAAP Reported Information to Non-GAAP Information

(In millions of U.S. dollars)

(unaudited)

	Three months ended September 30					
	2021					
		Adjustments				
	GAAP Reported	**Interest, Taxes, Depreciation and Amortization**	**Stock-Based Compensation, Contingent Consideration and Other Adjustments**	**Non-GAAP**	**GAAP Reported**	**De Ar**
Cost of sales	**$ 103.5**	$ —	$ (4.3)	$ 99.2	**$ 188.8**	$
Research and development	**157.9**	(6.3)	(17.9)	133.7	**147.1**	
Selling, general and administrative	**183.3**	(5.1)	(26.9)	151.3	**179.5**	
Intangible asset amortization and contingent consideration	**17.2**	(15.4)	(1.8)	—	**17.4**	
Interest expense, net	**(2.0)**	2.0	—	—	**(5.6)**	
Benefit from income taxes	**(9.7)**	9.7	—	—	**(846.0)**	
GAAP Net Income (Loss) / Non-GAAP Income	**$ (36.5)**	$ 19.1	$ 50.9	$ 33.5	**$ 784.8**	$

Nine months ended September 30

	2021					D
		Adjustments				
	GAAP Reported	Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non-GAAP	GAAP Reported	Ar
Cost of sales	**$ 350.8**	$ —	$ (16.9)	$ 333.9	**$ 398.1**	$
Research and development	**467.7**	(20.9)	(56.2)	390.6	**471.4**	
Selling, general and administrative	**541.8**	(14.8)	(80.3)	446.7	**542.2**	
Intangible asset amortization and contingent consideration	**52.6**	(46.3)	(6.3)	—	**48.0**	
Gain on sale of nonfinancial assets	**—**	—	—	—	**(59.5)**	
Interest expense, net	**(2.8)**	2.8	—	—	**(11.0)**	
Benefit from income taxes	**(2.6)**	2.6	—	—	**(839.1)**	
GAAP Net Income (Loss) / Non-GAAP Income	**$ (6.2)**	$ 82.2	$ 159.7	$ 235.7	**$ 837.0**	$

SOURCE BioMarin Pharmaceutical Inc.
