BioMarin Announces Third Quarter 2022 Year-over-Year Total Revenue Growth of 24% (31% Excluding KUVAN®)

- VOXZOGO® $48 Million Contribution in the Quarter Results in BioMarin Raising Full-year 2022 VOXZOGO Net Product Revenue Guidance to Between $140 Million and $170 Million
- U.S. Biologics Application for ROCTAVIAN™ Accepted by U.S. Food and Drug Administration (FDA); Prescription Drug User Fee Act (PDUFA) Target Action Date is March 31, 2023; Commercial Launch of ROCTAVIAN Underway in the European Union (EU) Following August 24, 2022 Approval

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

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2022	2021	% Change	2022	2021	% Change
Total Revenues	$ 505.3	$ 408.7	24 %	$ 1,558.5	$ 1,396.5	12 %
Net Product Revenues Marketed by BioMarin (1)	$ 464.3	$ 369.5	26 %	$ 1,425.7	$ 1,245.8	14 %
VIMIZIM® Net Product Revenues	$ 155.5	$ 136.9	14 %	$ 511.7	$ 466.8	10 %
NAGLAZYME® Net Product Revenues	$ 99.5	$ 71.2	40 %	$ 343.3	$ 297.3	15 %
PALYNZIQ® Net Product Revenues	$ 66.2	$ 60.7	9 %	$ 182.7	$ 173.7	5 %
KUVAN Net Product Revenues	$ 57.0	$ 67.7	(16) %	$ 174.0	$ 217.3	(20) %
VOXZOGO Net Product Revenues	$ 48.3	$ 0.1	nm(2)	$ 102.3	$ 0.1	nm(2)
BRINEURA® Net Product Revenues	$ 37.8	$ 32.9	15 %	$ 111.7	$ 90.6	23 %
ALDURAZYME® Net Product Revenues	$ 29.0	$ 24.4	19 %	$ 90.8	$ 102.5	(11) %
GAAP Net Income (Loss)	$ (6.7)	$ (36.5)		$ 141.8	$ (6.2)	
GAAP Net Income (Loss) per Share – Basic	$ (0.04)	$ (0.20)		$ 0.77	$ (0.03)	
GAAP Net Income (Loss) per Share – Diluted	$ (0.04)	$ (0.20)		$ 0.75	$ (0.03)	
Non-GAAP Income (3)	$ 82.7	$ 33.5		$ 297.2	$ 235.7	

	September 30, 2022	December 31, 2021
Total cash, cash equivalents & investments	$ 1,646.1	$ 1,521.7

(1) Net Product Revenues Marketed by BioMarin is the sum of revenues from VIMIZIM, NAGLAZYME, PALYNZIQ, KUVAN, VOXZOGO and BRINEURA for the three and nine months ended September 30, 2022 and 2021, each calculated in accordance with Generally Accepted Accounting Principles in the United States (U.S. GAAP). Sanofi is BioMarin's sole customer for ALDURAZYME and is responsible for marketing and selling ALDURAZYME to third parties.

(2) Not meaningful

(3) 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 9 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.

SAN RAFAEL, Calif., Oct. 26, 2022 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) (BioMarin or the Company) today announced financial results for the third quarter ended September 30, 2022.

BioMarin Announces Third Quarter 2022 Year-over-year Total Revenue Growth of 24% (31% Excluding KUVAN)

"As anticipated, BioMarin is on-track to deliver double-digit revenue growth and profitability for the full-year 2022, underscored by our record year-to-date operating results. VOXZOGO demand is driving our financial performance and we expect additional launches in Japan and other global markets to further accelerate sales of this innovative product," said Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin. "Our third quarter and year-to-date performance not only showcased the continuing success of our VOXZOGO commercial launch, but also the European regulatory approval of ROCTAVIAN, the world's first gene therapy approved for the treatment of severe hemophilia A. The launch in the EU is underway

and, in the United States, the BLA for ROCTAVIAN was accepted by the FDA with an assigned PDUFA target action date of March 31, 2023. With two key product approvals and commercial launches over the past 12-months, the foundation of our 5-year strategic plan is in place."

Financial Highlights:

- **Total Revenues** for the third quarter of 2022 were $505.3 million, an increase of 24% compared to the same period in 2021 despite continued erosion of the U.S. KUVAN market, and incremental foreign exchange headwinds. The increase in Total Revenues was primarily attributed to the following:
  - Higher VOXZOGO commercial sales due to new patients initiating therapy globally following regulatory approvals by the European Medicines Agency (EMA) and the FDA in the third and fourth quarters of 2021, respectively and
  - Higher NAGLAZYME and VIMIZIM product revenues primarily driven by the timing of orders in countries that place large government orders, particularly in Europe and Latin America and new patients initiating therapy in Europe and the Middle East; partially offset by
  - Lower KUVAN product revenues primarily due to generic competition as a result of the loss of exclusivity in the U.S., consistent with expectations.
- **GAAP Net Loss** decreased to $6.7 million for the third quarter of 2022 compared to GAAP Net Loss of $36.5 million for the same period in 2021. The decrease was primarily related to higher gross profit driven by increased sales volume, partially offset by higher selling, general and administrative (SG&A) expenses and a higher tax provision. The increase in SG&A expenses was largely due to higher costs to support the commercial launch of VOXZOGO and ROCTAVIAN, higher foreign currency exchange losses and severance costs associated with the Company's organizational redesign announced in October 2022. The increase to the tax provision was primarily attributed to higher year-to-date income driven by increased gross profits and the net gain on the sale of the Priority Review Voucher during the first quarter of 2022.
- **Non-GAAP Income** increased to $82.7 million for the third quarter of 2022 compared to Non-GAAP Income of $33.5 million for the same period in 2021 driven by higher gross profit due to increased sales volume partially offset by higher SG&A expenses largely driven by higher costs to support the commercial launch of VOXZOGO and ROCTAVIAN and higher foreign currency losses.

New Product Approvals and Launches (ROCTAVIAN and VOXZOGO)

- Following EMA approval in the quarter, the commercial launch of ROCTAVIAN is now underway. It is estimated that approximately 20,000 adults are affected by severe hemophilia A across more than 70 countries in Europe, the Middle East, and Africa. Of the 8,000 adults with severe hemophilia A in the 24 countries within BioMarin's footprint covered by the EMA approval, there are an estimated 3,200 patients who are indicated for ROCTAVIAN based on the current label.
- To determine eligibility for ROCTAVIAN, treating physicians in countries covered by the EMA approval can use a companion diagnostic (CDx) test to ensure that patients do not have pre-existing antibodies to AAV5. The CDx test is CE-marked and designed to ensure the highest safety standards for use in determining patient eligibility for treatment with ROCTAVIAN.
- On October 12, 2022, BioMarin's resubmission of the BLA for ROCTAVIAN was accepted by the FDA with a PDUFA target action date of March 31, 2023. The FDA recently communicated plans to hold an advisory committee meeting but has yet to provide a date. If approved, ROCTAVIAN would be the first gene therapy in the U.S. for the treatment of severe hemophilia A.
- At present, in the U.S. the Premarket Approval (PMA) application is under review at the Center for Devices and Radiological Health to support contemporaneous approval of a CDx along with the ROCTAVIAN BLA.
- The global expansion of VOXZOGO is actively underway, with market access and reimbursement progressing as anticipated. As of September 30, 2022, there were 29 active markets contributing to VOXZOGO sales with an estimated 713 children being treated, as compared to an estimated 446 children as of June 30, 2022.
- In the quarter, VOXZOGO became commercially available in Japan resulting in meaningful contributions from the early launch. Japan accounts for approximately half of the 1,500 patient opportunity in the Asia-Pacific region.

Mid-stage Product Life Cycle Expansion Opportunities (VOXZOGO and ROCTAVIAN)

- During the quarter, the Company held discussions with global regulatory health authorities regarding the favorable results from the Phase 2 randomized, double-blind, placebo-controlled VOXZOGO study in infants and young children up to five years of age with achondroplasia. Based on these interactions, BioMarin intends to submit supplemental marketing applications by the end of 2022 in the U.S. and EU to expand access to VOXZOGO treatment for this younger age group.
- Product expansion opportunities with ROCTAVIAN are supported by a number of clinical studies currently underway. The Phase 3b study to evaluate ROCTAVIAN with prophylactic corticosteroids has completed enrollment and is expected to read-out in early 2023. Two additional studies, one investigating ROCTAVIAN treatment in those with active or prior inhibitors, as well as one study investigating ROCTAVIAN in people with pre-existing antibodies against AAV5.

Earlier-stage Development Portfolio (BMN 255, BMN 331, BMN 351, BMN 349, BMN 293 (DiNA-001))

- BMN 255 for primary hyperoxaluria, a prognostic factor for chronic renal disease: The Company is proceeding with the multi-ascending dose phase of the First-in-Human study with BMN 255. BioMarin believes 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): Dosing continues in the Phase 1/2 HAERMONY study to evaluate BMN 331, an investigational AAV5-mediated gene therapy for people living with HAE, including dose escalation to the 6e13vg/kg dose, which our non-clinical studies project to provide therapeutic levels of C1-inhibitor.
- BMN 351 for Duchenne Muscular Dystrophy (DMD): Investigational New Drug application (IND)-enabling studies continue with BMN 351, an antisense oligonucleotide therapy for individuals with exon 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 tolerability in preclinical models. Preclinical data suggest that restored expression of near-full-length dystrophin protein at levels of up to 40% will convert phenotypes from rapid loss to durable preservation of strength and ambulation. The IND is expected to be activated in early 2023 to enable initiation of the clinical phase of development.
- BMN 349 for alpha-1 antitrypsin deficiency: Preclinical studies have demonstrated that BMN 349 is an orally bioavailable, small molecule that is titratable with rapid onset and high potency and efficacy. Preclinical results have strong implications for potential improvement of current management, particularly for severe liver disease requiring rapid action. IND enabling studies are well-underway and BioMarin's goal is to file an IND for BMN 349 in the second half of 2023.
- BMN 293 (formerly DiNA-001) for MYBPC3 hypertrophic cardiomyopathy (HCM): Preclinical studies are underway with BMN 293 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 cardiac hypertrophy, improve relaxation kinetics and potentially alleviate functional deficits in individuals suffering from cardiomyopathy. BioMarin's goal is to file an IND for BMN 293 in 2023.

2022 Full-Year Financial Guidance (in millions, except %)

Item	Provided August 3, 2022			Revised October 26, 2022		
Total Revenues	$2,060	to	$2,160	Unchanged		
VIMIZIM Net Product Revenues	$655	to	$700	Unchanged		
NAGLAZYME Net Product Revenues	$415	to	$450	Unchanged		
PALYNZIQ Net Product Revenues	$250	to	$275	Unchanged		
KUVAN Net Product Revenues	$210	to	$235	Unchanged		
BRINEURA Net Product Revenues	$145	to	$160	Unchanged		
VOXZOGO Net Product Revenues	$130	to	$160	$140	to	$170
Cost of Sales (% of Total Revenues)	22.5 %	to	24.5 %	Unchanged		
Research and Development Expense	$665	to	$715	$650	to	$700
Selling, General and Administrative Expense	$790	to	$840	$800	to	$850
GAAP Net Income	$105	to	$145	Unchanged		
Non-GAAP Income (1)	$350	to	$390	Unchanged		

(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 9 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 2022 financial results today, Wednesday, October 26, 2022 at 4:30 p.m. ET. This event can be accessed through this link or on the investor section of the BioMarin website at www.biomarin.com.

U.S./Canada Dial-in Number: 800-231-0316	Replay Dial-in Number: 800-645-7964
International Dial-in Number: 314-696-0504	Replay International Dial-in Number: 757-849-6722
No Conference ID	Conference ID: 2361#

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 seven commercial products and multiple clinical and preclinical product candidates for the treatment of various diseases. 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 2022; cash flows from operating activities; the timing of orders for commercial products; 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) the outcome of BioMarin's BLA resubmission for ROCTAVIAN to the FDA (as well as the outcome of the PMA application submitted for contemporaneous approval of the related CDx test), (ii) the results from clinical studies regarding product expansion opportunities for ROCTAVIAN, (iii) BioMarin's plans to submit supplemental marketing applications in the U.S. and EU to expand access to VOXZOGO for infants and young children up to five years of age with achondroplasia by the end of 2022, (v) BioMarin's anticipated IND activation for BMN 351 in early 2023, (vi) BioMarin's anticipated IND submission for BMN 349 in the second half of 2023, and (vii) BioMarin's goal to file an IND for BMN 293 in 2023; the potential approval and commercialization of BioMarin's product candidates, including ROCTAVIAN for the treatment of severe hemophilia A, and the timing of such approval decisions and product launches, including (i) the anticipated start of commercial sales of VOXZOGO in additional countries, and (ii) the duration of the FDA's review procedure of our BLA resubmission for ROCTAVIAN, including the possibility that the FDA determines more review time is necessary based on the number of data read-outs that will emerge during the procedure; and the expected benefits and availability of BioMarin's product candidates; the anticipated benefits of BioMarin's organizational redesign plan announced in October 2022; and potential growth opportunities and trends, including that BioMarin expects (i) double-digit growth in revenues and profitability in 2022, and (ii) increasing access to VOXZOGO as the product launch continues in future quarters, including BioMarin's expectation that the addition of Japan and other global markets will increase the pace of uptake in demand for VOXZOGO.

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; successful implementation of BioMarin's organizational redesign plan announced in October 2022; 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, 2022 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®, BRI

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED BALANCE SHEETS

September 30, 2022 and December 31, 2021

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

	September 30, 2022	December 31, 2021 [1]
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 761,515	$ 587,276
Short-term investments	512,253	426,599

Accounts receivable, net	419,622	373,399
Inventory	839,460	776,669
Other current assets	149,851	110,442
Total current assets	2,682,701	2,274,385
Noncurrent assets:		
Long-term investments	372,302	507,793
Property, plant and equipment, net	1,051,821	1,035,461
Intangible assets, net	354,024	388,652
Goodwill	196,199	196,199
Deferred tax assets	1,455,205	1,450,161
Other assets	151,788	152,121
Total assets	$ 6,264,040	$ 6,004,772
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 492,717	$ 498,265
Short-term contingent consideration	28,303	48,232
Total current liabilities	521,020	546,497
Noncurrent liabilities:		
Long-term convertible debt, net	1,082,033	1,079,077
Long-term contingent consideration	—	15,167
Other long-term liabilities	92,473	98,361
Total liabilities	1,695,526	1,739,102
Stockholders' equity:		
Common stock, $0.001 par value: 500,000,000 shares authorized; 185,824,253 and 183,912,514 shares issued and outstanding, respectively	186	184
Additional paid-in capital	5,335,853	5,191,502
Company common stock held by the Nonqualified Deferred Compensation Plan	(9,325)	(9,689)
Accumulated other comprehensive income	30,749	14,432
Accumulated deficit	(788,949)	(930,759)
Total stockholders' equity	4,568,514	4,265,670
Total liabilities and stockholders' equity	$ 6,264,040	$ 6,004,772

(1) During the third quarter of 2022, the Company identified and corrected an immaterial error related to its January 2020 sale of the worldwide rights of FIRDAPSE®. This immaterial error correction affected the previously issued condensed consolidated balance sheet at December 31, 2021. There was no impact to the condensed consolidated statements of operations for any of the periods presented.

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

Nine Months Ended September 30, 2022 and 2021

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

(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	**2021**	**2022**	**2021**
REVENUES:				
Net product revenues	$ 493,348	$ 393,840	$ 1,516,533	$ 1,348,279
Royalty and other revenues	11,996	14,902	41,968	48,186

Total revenues	505,344	408,742	1,558,501	1,396,465
OPERATING EXPENSES:				
Cost of sales	116,288	103,537	356,379	350,765
Research and development	157,829	157,869	476,855	467,701
Selling, general and administrative	216,816	183,333	608,270	541,812
Intangible asset amortization and contingent consideration	16,828	17,222	50,935	52,648
Gain on sale of nonfinancial assets, net	—	—	(108,000)	—
Total operating expenses	507,761	461,961	1,384,439	1,412,926
INCOME FROM OPERATIONS	(2,417)	(53,219)	174,062	(16,461)
Interest income	4,999	1,827	9,324	8,737
Interest expense	(4,679)	(3,870)	(12,344)	(11,491)
Other income (expense), net	193	9,102	(3,908)	10,439
INCOME (LOSS) BEFORE INCOME TAXES	(1,904)	(46,160)	167,134	(8,776)
Provision for (benefit from) income taxes	4,748	(9,666)	25,324	(2,594)
NET INCOME (LOSS)	$ (6,652)	$ (36,494)	$ 141,810	$ (6,182)
NET INCOME (LOSS) PER SHARE, BASIC	$ (0.04)	$ (0.20)	$ 0.77	$ (0.03)
NET INCOME (LOSS) PER SHARE, DILUTED	$ (0.04)	$ (0.20)	$ 0.75	$ (0.03)
Weighted average common shares outstanding, basic	185,597	183,214	185,009	182,616
Weighted average common shares outstanding, diluted	185,597	183,214	192,252	182,616

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS

Nine Months Ended September 30, 2022 and 2021

(In thousands of U.S. dollars)

(unaudited)

	Nine Months Ended September 30,	
	2022	**2021**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net income (loss)	$ 141,810	$ (6,182)
Adjustments to reconcile net income (loss) to net cash provided by operating activities:		
Depreciation and amortization	77,416	82,053
Non-cash interest expense	3,089	3,114
Amortization of premium on investments	3,741	3,279
Stock-based compensation	149,574	153,372
Gain on sale of nonfinancial assets, net	(108,000)	—
Deferred income taxes	(743)	(12,020)
Unrealized foreign exchange gain	(16,075)	(1,347)
Non-cash changes in the fair value of contingent consideration	2,243	6,254
Other	(700)	(1,317)
Changes in operating assets and liabilities:		
Accounts receivable, net	(53,752)	65,513
Inventory	(27,419)	(19,125)
Other current assets	(8,558)	27,029
Other assets	12,140	(407)
Accounts payable and other short-term liabilities	(2,398)	(7,129)

Other long-term liabilities	(3,252)	269
Net cash provided by operating activities	169,116	293,356
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of property, plant and equipment	(85,271)	(66,840)
Maturities and sales of investments	477,244	502,112
Purchases of investments	(457,382)	(737,144)
Proceeds from sale of nonfinancial assets	110,000	—
Purchase of intangible assets	(9,910)	(8,026)
Other	—	(994)
Net cash provided by (used in) investing activities	34,681	(310,892)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from exercises of awards under equity incentive plans	43,866	32,877
Taxes paid related to net share settlement of equity awards	(50,696)	(44,428)
Payment of contingent consideration	(21,054)	—
Principal repayments of financing leases	(1,635)	(2,492)
Other	—	(401)
Net cash used in financing activities	(29,519)	(14,444)
Effect of exchange rate changes on cash	(39)	(35)
NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS	174,239	(32,015)
Cash and cash equivalents:		
Beginning of period	$ 587,276	$ 649,158
End of period	$ 761,515	$ 617,143

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 to Non-GAAP Income:

Reconciliation of GAAP Net Income to Non-GAAP Income

(In millions of U.S. dollars)

(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,		Guidance [2] Year Ending
	2022	**2021**	**2022**	**2021**	**December 31, 2022**
GAAP Net Income (Loss)	**$ (6.7)**	**$ (36.5)**	**$ 141.8**	**$ (6.2)**	**$ 105.0 to $ 145.0**
Interest income (expense), net	(0.3)	2.0	3.0	2.8	4.0
Provision for (benefit from) income taxes	4.7	(9.7)	25.3	(2.6)	27.0
Depreciation expense	8.7	11.4	29.9	35.7	40.0
Amortization expense	15.9	15.4	47.1	46.3	63.0
Stock-based compensation expense	54.7	49.1	149.5	153.4	192.0
Contingent consideration expense	0.9	1.8	3.8	6.3	4.0
Severance and reorganization costs[1]	4.8	—	4.8	—	23.0
Gain on sale of nonfinancial assets, net	—	—	(108.0)	—	(108.0)
Non-GAAP Income	$ 82.7	$ 33.5	$ 297.2	$ 235.7	$ 350.0 to $ 390.0

(1) Represents third quarter 2022 severance and employee termination benefit charge related to the Company's organizational redesign announced in October 2022. The Company expects to incur total estimated pre-tax charges of approximately $20.0 million to $25.0 million in 2022.

(2) The adjustments/reconciling items included in the Guidance Year Ending December 31, 2022 column are presented to facilitate the reconciliation of Non-GAAP Income to its closest GAAP financial metric, GAAP Net Income (Loss). The Company notes that the specific amounts included in each reconciling line item above represent approximations of the underlying adjustments from GAAP Net Income (Loss) to Non-GAAP Income, and that actual 2022 results for each reconciling line item may be different, in some cases materially, than the amounts listed above as a result of uncertainty regarding, and the potential variability of, those items.

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,					
	2022					
		Adjustments				
	GAAP Reported	**Interest, Taxes, Depreciation and Amortization**	**Stock-Based Compensation, Contingent Consideration and Other Adjustments**	**Non-GAAP**	**GAAP Reported**	**Intere Taxe Depreci and Amortiz**
Cost of sales	**$ 116.3**	$ —	$ (4.4)	$ 111.9	**$ 103.5**	$
Research and development	**157.8**	(4.7)	(17.1)	136.0	**157.9**	
Selling, general and administrative	**216.8**	(4.0)	(38.0)	174.8	**183.3**	
Intangible asset amortization and contingent consideration	**16.8**	(15.9)	(0.9)	—	**17.2**	(
Gain on sale of nonfinancial assets	**—**	—	—	—	**—**	
Interest income (expense), net	**0.3**	(0.3)	—	—	**(2.0)**	

Provision for (benefit from) income taxes	**4.7**	(4.7)	—	—	**(9.7)**	
GAAP Net Income (Loss) /Non-GAAP Income	**$ (6.7)**	$ 29.0	$ 60.4	$ 82.7	**$ (36.5)**	$

	Nine Months Ended September 30,					
	2022					
		Adjustments				
	GAAP Reported	**Interest, Taxes, Depreciation and Amortization**	**Stock-Based Compensation, Contingent Consideration and Other Adjustments**	**Non-GAAP**	**GAAP Reported**	**Intere Taxe Depreci and Amortiz**
Cost of sales	**$ 356.4**	$ —	$ (13.4)	$ 343.0	**$ 350.8**	$
Research and development	**476.9**	(17.4)	(47.9)	411.6	**467.7**	(
Selling, general and administrative	**608.3**	(12.5)	(93.0)	502.8	**541.8**	(
Intangible asset amortization and contingent consideration	**50.9**	(47.1)	(3.8)	—	**52.6**	(
Gain on sale of nonfinancial assets, net	**(108.0)**	—	108.0	—	**—**	
Interest expense, net	**(3.0)**	3.0	—	—	**(2.8)**	
Provision for (benefit from) income taxes	**25.3**	(25.3)	—	—	**(2.6)**	
GAAP Net Income (Loss) / Non-GAAP Income	**$ 141.8**	$ 105.3	$ 50.1	$ 297.2	**$ (6.2)**	$

Contact:

Investors:
Traci McCarty
BioMarin Pharmaceutical Inc.
(415) 455-7558

Media:
Debra Charlesworth
BioMarin Pharmaceutical Inc.
(415) 455-7451

SOURCE BioMarin Pharmaceutical Inc.
