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**BioMarin Announces Record Fourth Quarter and Full Year 2022 Total Revenues Driven by Strong Global Demand for VOXZOGO® and Steady Growth of Enzyme Business**

- VOXZOGO \$169 Million Contribution Drives Record Full Year 2022 Total Revenues of \$2.1 Billion
- In 2023, More than 15% Growth in Total Revenues and Approximately 30% Growth in Net Income Expected Based on Mid-point of Today's Full-year Guidance; VOXZOGO Revenues Expected to More than Double
- ROCTAVIAN™ European Commercial Launch and U.S. Launch Preparations Both Underway; 3-year Analysis from Phase 3 ROCTAVIAN Study in Adults with Severe Hemophilia A Recently Submitted to the FDA

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

	Three Months Ended December 31,			Twelve Months Ended December 31,		
	2022	2021	% Change	2022	2021	% Change
Total Revenues	\$ 537.5	\$ 449.8	19 %	\$ 2,096.0	\$ 1,846.3	14 %
Net Product Revenues Marketed by BioMarin <sup>(1)</sup>	\$ 487.9	\$ 414.9	18 %	\$ 1,913.6	\$ 1,660.7	15 %
VIMIZIM® Net Product Revenues	\$ 152.1	\$ 156.3	(3)%	\$ 663.8	\$ 623.1	7 %
NAGLAZYME® Net Product Revenues	\$ 100.5	\$ 83.1	21 %	\$ 443.8	\$ 380.4	17 %
PALYNZIQ® Net Product Revenues	\$ 72.3	\$ 63.8	13 %	\$ 255.0	\$ 237.5	7 %
VOXZOGO Net Product Revenues	\$ 66.8	\$ 5.8	1,052 %	\$ 169.1	\$ 5.9	2,766 %
KUVAN® Net Product Revenues	\$ 53.6	\$ 68.5	(22)%	\$ 227.6	\$ 285.8	(20)%
BRINEURA® Net Product Revenues	\$ 42.6	\$ 37.4	14 %	\$ 154.3	\$ 128.0	21 %
ALDURAZYME® Net Product Revenues	\$ 37.6	\$ 20.3	85 %	\$ 128.4	\$ 122.8	5 %
GAAP Net Income (Loss)	\$ (0.2)	\$ (57.9)		\$ 141.6	\$ (64.1)	
GAAP Earnings (Loss) per Share – Basic	\$ (0.00)	\$ (0.32)		\$ 0.76	\$ (0.35)	
GAAP Earnings (Loss) per Share – Diluted	\$ (0.00)	\$ (0.32)		\$ 0.75	\$ (0.35)	
Non-GAAP Income <sup>(2)</sup>	\$ 67.4	\$ 7.1		\$ 364.6	\$ 242.8	

	December 31, 2022	December 31, 2021
Total cash, cash equivalents & investments	\$ 1,625.4	\$ 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 twelve months ended December 31, 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) Non-GAAP Income for the historical periods presented is defined by the Company as reported GAAP Net Income/Loss, excluding net interest income (expense), provision for (benefit from) income taxes, depreciation expense, amortization expense, stock-based compensation expense, contingent consideration expense and, in certain periods, certain other specified items. Refer to Non-GAAP Information beginning on page [10](#) of this press release for a complete discussion of the Company's Non-GAAP financial information and reconciliations to the comparable information reported under U.S. GAAP.

SAN RAFAEL, Calif., February 27, 2023 – BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) (BioMarin or the Company) today announced financial results for the fourth quarter and full year ended December 31, 2022.

"As expected, in 2022 BioMarin delivered double-digit revenue growth and profitability for the full-year driven by the strong global launch of VOXZOGO, consistent growth of our enzyme business and continued focus on operational excellence," said Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin. "Our record-setting 2023 outlook underscores BioMarin's proven and fully-scaled development and commercial capabilities and attention to sustainable growth. With global market expansion of VOXZOGO well underway, we turn our focus to the European commercial launch of ROCTAVIAN, the world's first gene therapy approved for the treatment of severe hemophilia A. Our team in Germany is working with the leading hemophilia centers of excellence to drive awareness and uptake of ROCTAVIAN, now that it is commercially available. In the United States, we are actively preparing for the launch of ROCTAVIAN upon potential approval this year. We are encouraged by the level of interest from U.S. adult hemophilia A patients seeking information about ROCTAVIAN and are pleased that roughly 300 people have engaged directly with BioMarin to learn more. Acknowledging that many of these people may not be eligible for treatment with ROCTAVIAN, we are glad to see this level of engagement with the bleeding disorders community."

### **Financial Highlights:**

- **Total Revenues** for the fourth quarter of 2022 were \$537.5 million, an increase of 19% compared to the same period in 2021 despite continued erosion of the U.S. KUVAN market. The increase in Total Revenues was primarily attributed to the following:
  - Higher VOXZOGO commercial sales due to continued global market expansion and rapid patient uptake following regulatory approvals in late 2021 and early 2022,
  - Higher NAGLAZYME product revenues primarily driven by new patients initiating therapy and the timing of orders in countries that place large government orders, particularly in Europe and the Middle East, and
  - Higher ALDURAZYME product revenues primarily due to the timing of order fulfillment to Sanofi. BioMarin ALDURAZYME revenues are driven by the timing of when the product is released and control is transferred to Sanofi,
  - Lower KUVAN product revenues primarily due to generic competition as a result of the loss of market exclusivity in the U.S., consistent with expectations.
- **GAAP Net Loss** decreased to \$0.2 million for the fourth quarter of 2022 compared to GAAP Net Loss of \$57.9 million for the same period in 2021. The decreased net loss was primarily related to higher gross profit, driven by increased sales volume. This was partially offset by higher selling, general and administrative (SG&A) and research and development (R&D) expenses. The increase in SG&A expenses was largely due to severance costs associated with the Company's organizational redesign announced in October 2022 and higher costs to support the commercial launch of VOXZOGO and ROCTAVIAN in the EU. The increase in R&D expenses was primarily attributed to higher spend for programs in our earlier-stage development portfolio.

- **Non-GAAP Income** increased to \$67.4 million for the fourth quarter of 2022 compared to Non-GAAP Income of \$7.1 million for the same period in 2021 driven by higher gross profit due to increased sales volume partially offset by higher SG&A and R&D expenses for the same reasons noted above.

### ***New Product Approvals and Launches (ROCTAVIAN and VOXZOGO)***

- The European launch of ROCTAVIAN is underway following EMA approval in the third quarter of 2022. Since approval, BioMarin continues to collaborate with German health insurers to secure novel Outcomes Based Agreements (OBAs) to enable access to ROCTAVIAN treatment. The first OBA has been completed, allowing for a significant percentage of people in Germany affected by severe hemophilia A to pursue treatment with ROCTAVIAN. Patient testing to determine eligibility for ROCTAVIAN treatment is ongoing throughout Germany.
- BioMarin's Biologics License Application (BLA) for ROCTAVIAN is currently under review by the U.S. Food and Drug Administration (FDA) with a PDUFA target action date of March 31, 2023, subject to a potential three-month extension, if the FDA deems necessary during the review procedure. The Company recently submitted to the FDA positive results from three or more years of follow up from its ongoing global Phase 3 GENE8-1 study of ROCTAVIAN, the largest and longest global Phase 3 study to date for any gene therapy in hemophilia with 134 participants. As part of the ongoing review, the FDA completed the Pre-License Inspection of the Company's dedicated gene therapy facility in December 2022. BioMarin has provided responses to the comments and observations received at the close of the FDA inspection, and believes all are addressable. Also 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.
- Today, the Company provided full-year 2023 ROCTAVIAN guidance of between \$100 million to \$200 million. The estimated range acknowledges the inherent uncertainties of the global launch during 2023, and assumes contributions from Germany, the United States, if approved, with the amount dependent on potential approval timing, and small numbers of patients in other markets.
- The global expansion of VOXZOGO continues, with market access and reimbursement activities progressing, as anticipated. As of the end of January 2023, an estimated 1,264 children with achondroplasia were being treated with VOXZOGO. Treated children are included under the currently approved age ranges in Europe, 2 years old and older, the United States, for children 5 years old and older, and in Japan, approved for all ages from birth. There were 32 active markets contributing to VOXZOGO commercial expansion including the United States, Europe, Japan, Canada, Australia and Brazil.

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

- During the fourth quarter, BioMarin submitted supplemental marketing applications in the U.S. and EU to expand VOXZOGO access to younger age groups, based on favorable results from a Phase 2 study in infants and young children. In January 2023, the European Medicines agency validated BioMarin's application for extension of indications for VOXZOGO for the treatment of children under the age of two. The Company expects action by U.S. and EU health authorities on the applications in the second half of 2023. If age expansions are accepted, more than 1,000 additional children will be eligible for VOXZOGO treatment in the U.S. and Europe.
- Product expansion opportunities with ROCTAVIAN are supported by a number of clinical studies currently underway. Two additional studies are ongoing, 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))**

- BioMarin plans to showcase progress across its earlier-stage development pipeline at R&D Day in New York City on September 12, 2023. Invitations to the event will be circulated in June.
- BMN 255 for hyperoxaluria in chronic liver disease: The Company has concluded the multi-ascending dose phase of the First-in-Human study with BMN 255. In January 2023, BioMarin shared early data that demonstrated a rapid and potent increase in plasma glycolate following treatment with BMN 255, which is predicted to have a profound reduction in oxalate excretion in patients. BioMarin now plans to initiate and fully enroll an expanded study in patients with chronic liver disease and hyperoxaluria in 2023. The Company believes the availability of a potent, orally bioavailable, small molecule like BMN 255 may be able to significantly reduce disease and treatment burden in a patient population with significant unmet need.
- 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. In January 2023, BioMarin shared that the first participant treated with the 6e13vg/kg dose demonstrated C1-Inhibitor levels that were approaching the therapeutically relevant range. A second participant is scheduled for dosing at the 6e13vg/kg dose level in the coming weeks.
- 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. BioMarin is working towards beginning clinical studies with BMN 351 in 2023.
- BMN 349 for alpha-1 antitrypsin deficiency: Preclinical studies have demonstrated that BMN 349 is an orally bioavailable, small molecule that in preclinical studies has demonstrated that it 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 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 platform company that develops organ specific delivery of novel gene therapies to treat rare genetic cardiac and renal diseases. Mutations in the MYBPC3 gene 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 the second half of 2023.

### **Change in Non-GAAP Measures Beginning in 2023**

Beginning with the first quarter of 2023, the Company defines Non-GAAP Income as GAAP Net Income excluding amortization of intangible assets, stock-based compensation expense, and certain other specified items. Reflecting this change in the Company's full year 2022 financial results as detailed above would have lowered the Company's full year 2022 Non-GAAP Income by \$73.8 million and its full year 2022 Non-GAAP diluted earnings per share (EPS) by \$0.38. The Company is also introducing a new Non-GAAP financial measure, Non-GAAP Diluted EPS, which is defined as Non-GAAP Income divided by Non-GAAP diluted shares outstanding. Refer to page 10 of this press release for a complete discussion of the Company's current Non-GAAP financial information and reconciliations to comparable information reported under U.S. GAAP.

**2023 Full-Year Financial Guidance (in millions, except % and EPS amounts)**

Item	2023 Guidance		
Total Revenues	\$2,375	to	\$2,500
Enzyme Product Revenues <sup>(1)</sup>	\$1,700	to	\$1,850
ROCTAVIAN Revenues	\$100	to	\$200
VOXZOGO Revenues	\$330	to	\$380
Gross Profit %	77.5%	to	79%
R&D % of Revenue	30%	to	32%
SG&A % of Revenue	36%	to	38%
GAAP Net Income	\$155	to	\$205
GAAP Diluted EPS	\$0.78	to	\$1.03
Non-GAAP Income (new method)	\$360	to	\$410
Non-GAAP Diluted EPS (new method)	\$1.80	to	\$2.05

(1) Enzyme Products include ALDURAZYME, VIMIZIM, NAGLAZYME, BRINEURA, and PALYNZIQ.

The full-year 2023 ROCTAVIAN revenue guidance range, provided above, represents global revenue estimates and assumes a U.S. approval in 2023, regardless of approval timing.

BioMarin will host a conference call and webcast to discuss fourth quarter and full year 2022 financial results today, Monday, February 27, 2023 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.biomin.com](http://www.biomin.com).

U.S./Canada Dial-in Number: 800-831-4163	Replay Dial-in Number: 800-645-7964
International Dial-in Number: 213-992-4616	Replay International Dial-in Number: 757-849-6722
No Conference ID	Conference ID: 9184#

**About BioMarin**

Founded in 1997, BioMarin is a global biotechnology company dedicated to transforming lives through genetic discovery. The Company develops and commercializes targeted therapies that address the root cause of genetic conditions. BioMarin's robust research and development capabilities have resulted in multiple innovative commercial therapies for patients with rare genetic disorders. The Company's distinctive approach to drug discovery has produced a diverse pipeline of commercial, clinical, and pre-clinical candidates that address a significant unmet medical need, have well-understood biology, and provide an opportunity to be first-to-market or offer a substantial benefit over existing treatment options. For additional information, please visit [www.biomin.com](http://www.biomin.com).

## Forward-Looking Statements

This press release and the associated conference call and webcast contain forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc. (BioMarin), including, without limitation, statements about: the expectations of Total Revenues, Net Product Revenues, Research and Development Expense, Selling, General and Administrative Expense, Cost of Sales, GAAP Net Income, Non-GAAP Income, Diluted GAAP EPS and Dilutive Non-GAAP EPS and other specified income statement guidance for the full-year 2023; 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 initiate and fully enroll an expanded study of BMN 255 in 2023 (iv) BioMarin's expectation to begin clinical studies with BMN 351 in the middle of 2023, (v) BioMarin's anticipated IND submission for BMN 349 in the second half of 2023, and (vi) BioMarin's goal to file an IND for BMN 293 in the second half of 2023; the potential approval and commercialization of BioMarin's product candidates, including ROCTAVIAN for the treatment of severe hemophilia A in the U.S., and the timing of such approval decisions and product launches, including (i) the anticipated start of commercial sales of VOXZOGO in additional countries, (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 (iii) BioMarin's expectation that U.S. and EU health authorities take action on its supplemental marketing applications for VOXZOGO in the second half of 2023 and the number of additional children that will be eligible for VOXZOGO if such age expansions are accepted and (iv) the level of interest about ROCTAVIAN from U.S. adult hemophilia A patients, including the number of people engaging with BioMarin to learn more; 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 increasing access to VOXZOGO as the product launch continues in future quarters, including BioMarin's expectation that the addition of Japan, Canada, Australia, Brazil and other global markets will increase the pace of uptake in demand for VOXZOGO and double sales in 2023.

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, impacts of macroeconomic and other external factors on BioMarin's operations; results and timing of current and planned 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; the market for each of these products; actual sales of BioMarin's commercial products; 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 September 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®, BRINEURA®, KUVAN®, NAGLAZYME®, PALYNZIQ®, VIMIZIM® and VOXZOGO® are registered trademarks of BioMarin Pharmaceutical Inc., or its affiliates. ROCTAVIAN™ is a trademark of BioMarin Pharmaceutical Inc. ALDURAZYME® is a registered trademark of BioMarin/Genzyme LLC. All other brand names and service marks, trademarks and other trade names appearing in this release are the property of their respective owners.

**BIOMARIN PHARMACEUTICAL INC.**  
**CONDENSED CONSOLIDATED BALANCE SHEETS**  
**December 31, 2022 and December 31, 2021**  
(In thousands of U.S. dollars, except per share amounts)

	<b>December 31, 2022</b>	<b>December 31, 2021 <sup>(1)</sup></b>
<b>ASSETS</b>	(unaudited)	
Current assets:		
Cash and cash equivalents	\$ 724,531	\$ 587,276
Short-term investments	567,006	426,599
Accounts receivable, net	461,316	373,399
Inventory	894,083	776,669
Other current assets	104,521	110,442
Total current assets	2,751,457	2,274,385
Noncurrent assets:		
Long-term investments	333,835	507,793
Property, plant and equipment, net	1,073,366	1,035,461
Intangible assets, net	338,569	388,652
Goodwill	196,199	196,199
Deferred tax assets	1,505,412	1,450,161
Other assets	176,236	152,121
Total assets	\$ 6,375,074	\$ 6,004,772
<b>LIABILITIES AND STOCKHOLDERS' EQUITY</b>		
Current liabilities:		
Accounts payable and accrued liabilities	\$ 572,959	\$ 498,265
Short-term contingent consideration	15,925	48,232
Total current liabilities	588,884	546,497
Noncurrent liabilities:		
Long-term convertible debt, net	1,083,019	1,079,077
Long-term contingent consideration	—	15,167
Other long-term liabilities	100,015	98,362
Total liabilities	1,771,918	1,739,103
Stockholders' equity:		
Common stock, \$0.001 par value: 500,000,000 shares authorized; 186,250,719 and 183,912,514 shares issued and outstanding, respectively	186	184
Additional paid-in capital	5,404,895	5,191,502
Company common stock held by the Nonqualified Deferred Compensation Plan	(8,859)	(9,689)
Accumulated other comprehensive income (loss)	(3,867)	14,432
Accumulated deficit	(789,199)	(930,760)
Total stockholders' equity	4,603,156	4,265,669
Total liabilities and stockholders' equity	\$ 6,375,074	\$ 6,004,772

(1) December 31, 2021 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, 2021, filed with the U.S. Securities and Exchange Commission (SEC) on February 25, 2022, except for balances related to the correction of an immaterial error that the Company identified and corrected in the third quarter 2022 related to its January 2020 sale of the worldwide rights of FIRDAPSE<sup>®</sup>. 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**  
**Three and Twelve Months Ended December 31, 2022 and 2021**  
(In thousands of U.S. dollars, except per share amounts)

	<b>Three Months Ended December 31,</b>		<b>Twelve Months Ended December 31,</b>	
	<b>2022</b>	<b>2021</b>	<b>2022</b>	<b>2021</b>
	(unaudited)	(unaudited)	(unaudited)	(unaudited)
<b>REVENUES:</b>				
Net product revenues	\$ 525,492	\$ 435,219	\$ 2,042,025	\$ 1,783,498
Royalty and other revenues	12,046	14,591	54,014	62,777
Total revenues	<u>537,538</u>	<u>449,810</u>	<u>2,096,039</u>	<u>1,846,275</u>
<b>OPERATING EXPENSES:</b>				
Cost of sales	127,290	119,750	483,669	470,515
Research and development	172,751	161,092	649,606	628,793
Selling, general and administrative	245,739	217,563	854,009	759,375
Intangible asset amortization and contingent consideration	16,258	17,285	67,193	69,933
Gain on sale of nonfinancial assets, net	—	—	(108,000)	—
Total operating expenses	<u>562,038</u>	<u>515,690</u>	<u>1,946,477</u>	<u>1,928,616</u>
<b>INCOME (LOSS) FROM OPERATIONS</b>	<u>(24,500)</u>	<u>(65,880)</u>	<u>149,562</u>	<u>(82,341)</u>
Interest income	8,710	1,745	18,034	10,482
Interest expense	(3,626)	(3,846)	(15,970)	(15,337)
Other income (expense), net	1,858	1,407	(2,050)	11,846
<b>INCOME (LOSS) BEFORE INCOME TAXES</b>	<u>(17,558)</u>	<u>(66,574)</u>	<u>149,576</u>	<u>(75,350)</u>
Provision for (benefit from) income taxes	<u>(17,309)</u>	<u>(8,676)</u>	<u>8,015</u>	<u>(11,270)</u>
<b>NET INCOME (LOSS)</b>	<u>\$ (249)</u>	<u>\$ (57,898)</u>	<u>\$ 141,561</u>	<u>\$ (64,080)</u>
<b>EARNINGS (LOSS) PER SHARE, BASIC</b>	<u>\$ (0.00)</u>	<u>\$ (0.32)</u>	<u>\$ 0.76</u>	<u>\$ (0.35)</u>
<b>EARNINGS (LOSS) PER SHARE, DILUTED</b>	<u>\$ (0.00)</u>	<u>\$ (0.32)</u>	<u>\$ 0.75</u>	<u>\$ (0.35)</u>
Weighted average common shares outstanding, basic	<u>186,028</u>	<u>183,554</u>	<u>185,266</u>	<u>182,852</u>
Weighted average common shares outstanding, diluted	<u>186,028</u>	<u>183,554</u>	<u>188,963</u>	<u>182,852</u>



**BIOMARIN PHARMACEUTICAL INC.**  
**CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS**  
**Twelve Months Ended December 31, 2022 and 2021**  
(In thousands of U.S. dollars)

	<b>Twelve Months Ended December</b>	
	<b>2022</b>	<b>2021</b>
	(unaudited)	
<b>CASH FLOWS FROM OPERATING ACTIVITIES:</b>		
Net income (loss)	\$ 141,561	\$ (64,080)
Adjustments to reconcile net income (loss) to net cash provided by operating activities:		
Depreciation and amortization	101,969	108,039
Non-cash interest expense	4,117	4,146
Amortization of premium on investments	3,043	5,155
Stock-based compensation	196,308	197,263
Gain on sale of nonfinancial assets, net	(108,000)	—
Deferred income taxes	(52,087)	(15,608)
Unrealized foreign exchange gain	(14,287)	(1,810)
Non-cash changes in the fair value of contingent consideration	1,704	8,026
Other	(2,043)	(2,629)
Changes in operating assets and liabilities:		
Accounts receivable, net	(82,033)	65,574
Inventory	(68,264)	(35,060)
Other current assets	7,822	29,760
Other assets	(19,859)	(6,593)
Accounts payable and other short-term liabilities	59,018	15,689
Other long-term liabilities	6,933	(3,336)
Net cash provided by operating activities	175,902	304,536
<b>CASH FLOWS FROM INVESTING ACTIVITIES:</b>		
Purchases of property, plant and equipment	(120,959)	(95,578)
Maturities and sales of investments	619,995	691,049
Purchases of investments	(611,809)	(937,143)
Proceeds from sale of nonfinancial assets	103,325	—
Purchase of intangible assets	(10,581)	(23,647)
Other	—	(994)
Net cash used in investing activities	(20,029)	(366,313)
<b>CASH FLOWS FROM FINANCING ACTIVITIES:</b>		
Proceeds from exercises of awards under equity incentive plans	69,333	49,194
Taxes paid related to net share settlement of equity awards	(54,283)	(45,805)
Payments of contingent consideration	(31,095)	—
Principal repayments of financing leases	(2,605)	(3,039)
Other	—	(398)
Net cash used in financing activities	(18,650)	(48)
Effect of exchange rate changes on cash	32	(57)
<b>NET INCREASE (DECREASE) IN CASH AND CASH EQUIVALENTS</b>	<b>\$ 137,255</b>	<b>\$ (61,882)</b>
Cash and cash equivalents:		
Beginning of period	\$ 587,276	\$ 649,158
End of period	\$ 724,531	\$ 587,276

## Non-GAAP Information

The results presented in this press release include both GAAP information and Non-GAAP information. As used in this release for 2022 and 2021 periods presented, Non-GAAP Income is defined by the Company as GAAP Net Income (Loss) excluding net interest income (expense), provision for (benefit from) income taxes, depreciation expense, amortization expense, stock-based compensation expense, contingent consideration expense and, in certain periods, certain other specified items, as detailed below when applicable. In addition, BioMarin includes in this press release the effects of these adjustments on certain components of GAAP Net Income (Loss) for each of the periods presented. In this regard, Non-GAAP Income and its components, including Non-GAAP Cost of Sales, Non-GAAP Research and Development expenses, Non-GAAP Selling, General and Administrative expense, Non-GAAP Intangible Asset Amortization and Contingent Consideration, Non-GAAP Gain on the Sale of Intangible Asset and Non-GAAP Benefit From Income Taxes are statement of operations line items prepared on the same basis as, and therefore components of, the overall Non-GAAP financial measure.

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 or 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 financial 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 financial measures; likewise, the Company may in the future cease to exclude items that it has historically excluded for purposes of its Non-GAAP financial measures. Because of the non-standardized definitions, the Non-GAAP financial 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 December 31,		Twelve Months Ended December 31,	
	2022	2021	2022	2021
<b>GAAP Net Income (Loss)</b>	<b>\$ (0.2)</b>	<b>\$ (57.9)</b>	<b>\$ 141.6</b>	<b>\$ (64.1)</b>
Interest income (expense), net	(5.1)	2.1	(2.1)	4.9
Provision for (benefit from) income taxes	(17.3)	(8.7)	8.0	(11.3)
Depreciation expense	8.7	10.4	38.6	46.1
Amortization expense	15.7	15.6	62.8	61.9
Stock-based compensation expense	46.8	43.9	196.3	197.3
Contingent consideration expense	0.6	1.7	4.4	8.0
Severance and reorganization costs <sup>(1)</sup>	18.2	—	23.0	—
Gain on sale of nonfinancial assets, net	—	—	(108.0)	—
Non-GAAP Income	<u>\$ 67.4</u>	<u>\$ 7.1</u>	<u>\$ 364.6</u>	<u>\$ 242.8</u>

(1) Represents 2022 severance and employee termination benefit charge related to the Company's organizational redesign announced in October 2022. The Company does not expect to incur significant charges related to this organizational redesign in 2023.

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 December 31,

	2022				2021			
	GAAP Reported	Adjustments			GAAP Reported	Adjustments		
		Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non-GAAP		Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non-GAAP
Cost of sales	\$ 127.3	\$ —	\$ (4.3)	\$ 123.0	\$ 119.8	\$ —	\$ (5.5)	\$ 114.3
Research and development	172.7	(4.5)	(13.8)	154.4	161.1	(6.1)	(11.0)	144.0
Selling, general and administrative	245.7	(4.2)	(46.9)	194.6	217.6	(4.3)	(27.4)	185.9
Intangible asset amortization and contingent consideration	16.3	(15.7)	(0.6)	—	17.3	(15.6)	(1.7)	—
Interest income (expense), net	5.1	(5.1)	—	—	(2.1)	2.1	—	—
Provision for (benefit from) income taxes	(17.3)	17.3	—	—	(8.7)	8.7	—	—
Net Income (Loss)	\$ (0.2)	\$ 2.0	\$ 65.6	\$ 67.4	\$ (57.9)	\$ 19.4	\$ 45.6	\$ 7.1

Twelve Months Ended December 31,

	2022				2021			
	GAAP Reported	Adjustments			GAAP Reported	Adjustments		
		Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non-GAAP		Interest, Taxes, Depreciation and Amortization	Stock-Based Compensation, Contingent Consideration and Other Adjustments	Non-GAAP
Cost of sales	\$ 483.7	\$ —	\$ (17.7)	\$ 466.0	\$ 470.5	\$ —	\$ (22.4)	\$ 448.1
Research and development	649.6	(21.9)	(61.7)	566.0	628.8	(27.0)	(67.2)	534.6
Selling, general and administrative	854.0	(16.7)	(139.9)	697.4	759.4	(19.1)	(107.7)	632.6
Intangible asset amortization and contingent consideration	67.2	(62.8)	(4.4)	—	69.9	(61.9)	(8.0)	—
Gain on sale of nonfinancial assets, net	(108.0)	—	108.0	—	—	—	—	—
Interest income (expense), net	2.1	(2.1)	—	—	(4.9)	4.9	—	—
Provision for (benefit from) income taxes	8.0	(8.0)	—	—	(11.3)	11.3	—	—
Net Income (Loss)	\$ 141.6	\$ 107.3	\$ 115.7	\$ 364.6	\$ (64.1)	\$ 101.6	\$ 205.3	\$ 242.8

The following table presents the reconciliation of 2023 guidance of GAAP Net Income and Diluted EPS to updated Non-GAAP Income and Non-GAAP Diluted EPS:

**Reconciliation of GAAP Net Income to Non-GAAP Income & Diluted EPS Guidance**  
(In millions, except per share amounts)  
(unaudited)

	Guidance Year Ending December 31, 2023 <sup>(1)</sup>		
	Net Income	Dilutive Shares	Diluted EPS
<b>GAAP Net Income and Diluted EPS</b>	<b>\$ 155.0 to \$ 205.0</b>	<b>200</b>	<b>\$0.78 to \$1.03</b>
Amortization expense	60.0		0.30
Stock-based compensation expense	207.0		1.04
Tax effect of adjustments <sup>(2)</sup>	(62.0)		(0.32)
Non-GAAP Income and Diluted EPS	<u>\$ 360.0 to \$ 410.0</u>	<u>200</u>	<u>\$ 1.80 to \$ 2.05</u>

(1) The adjustments/reconciling items included in the Guidance Year Ending December 31, 2023 column are presented to facilitate the reconciliation of Non-GAAP Income to its closest GAAP financial metric, GAAP Net Income. The Company notes that the specific amounts included in each reconciling line item above represent approximations of the underlying adjustments from GAAP Net Income to Non-GAAP Income, and that actual 2023 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.

(2) Income tax adjustments represent the estimated income tax impact of each pre-tax non-GAAP adjustment based on the applicable statutory income tax rate.

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