**BioMarin Announces First Quarter 2021 Financial Results and Corporate Updates**
***- Full-year 2021 Financial Guidance Reaffirmed***

***- Total Revenues Grew 9% in the First Quarter 2021 Compared to First Quarter 2020, Excluding Kuvan***

***- Regulatory Actions in Europe for Vosoritide and Valoctocogene Roxaparvovec Tracking as Expected; BioMarin Anticipates CHMP Opinion in June for Vosoritide and June Re-submission of MAA for Valoctocogene Roxaparvovec***

***- FDA Review of Vosoritide Proceeding; PDUFA Target Action Date Extended to November 20, 2021 to Provide Time for Review of Recently Submitted 2-year Phase 3 Results***

SAN RAFAEL, Calif., April 29, 2021 /PRNewswire/ --

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

| | Three Months Ended March 31, | | |
|---|---|---|---|
| | **2021** | **2020** | **% Change** |
| Total Revenues | $ 486.0 | $ 502.1 | (3) % |
| Net Product Revenues Marketed by BioMarin (1) | $ 417.8 | $ 433.3 | (4) % |
| Vimizim Net Product Revenues | $ 158.4 | $ 137.2 | 15 % |
| Naglazyme Net Product Revenues | $ 107.3 | $ 114.3 | (6) % |
| Kuvan Net Product Revenues | $ 70.8 | $ 122.0 | (42) % |
| Palynziq Net Product Revenues | $ 54.0 | $ 34.6 | 56 % |
| Brineura Net Product Revenues | $ 27.3 | $ 24.0 | 14 % |
| Aldurazyme Net Product Revenues | $ 50.0 | $ 55.7 | (10) % |
| GAAP Net Income | $ 17.4 | $ 81.4 | |
| GAAP Net Income per Share – Basic | $ 0.10 | $ 0.45 | |
| GAAP Net Income per Share – Diluted | $ 0.09 | $ 0.44 | |
| Non-GAAP Income (2) | $ 104.4 | $ 116.5 | |

| | **March 31, 2021** | **December 31, 2020** |
|---|---|---|
| Cash, cash equivalents and investments | $ 1,408.6 | $ 1,350.9 |

(1) Net Product Revenues Marketed by BioMarin is the sum of revenues from Vimizim, Kuvan, Naglazyme, Palynziq, Brineura and Firdapse, each calculated in accordance with Generally Accepted Accounting Principles in the United States (U.S. GAAP). Sanofi Genzyme (Genzyme) is BioMarin's sole customer for Aldurazyme and is responsible for marketing and selling Aldurazyme to third parties. Refer to page 9 for a table showing Net Product Revenues by product. In January 2020, BioMarin divested the Firdapse assets to a third party in a sale transaction. The sale is reflected in the Company's

consolidated financial statements for the first quarter ended March 31, 2020; as a result of the transaction BioMarin will not recognize Net Product Revenues from Firdapse in the future.

(2) Non-GAAP Income is defined by the Company as reported GAAP Net Income/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 first quarter ended March 31, 2021.

"We mark the start of 2021 with strong financial results, and a number of exciting regulatory decisions ahead this year," said Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin. "The potential approval in Europe this summer of vosoritide, the first potential therapeutic option for children with achondroplasia, will set the stage for our next significant phase of growth, especially considering the EMEA region is 3 times larger than the U.S. market. To date, the interactions with the review team at the European Medicines Agency have been very positive. Next, we plan to re-submit the application for valoctocogene roxaparvovec gene therapy, to treat hemophilia A, to the EMA in the second quarter, to be followed by potential approval of vosoritide in the United States later this year."

Mr. Bienaimé continued, "We are well positioned to deliver strong results for the full-year 2021, and reaffirm our financial guidance provided early this year. This takes into consideration the uneven ordering patterns in our dynamic and global commercial business, as well as continued uncertainty outside of the United States from COVID-19, and further underscores the essential nature of our products to the people who rely on them. Taken together, our financial strength, including positive cash flows from operations of $113.5 million in the first quarter, combined with near-term opportunities from potential new products and a robust early-stage pipeline, we believe that 2021 will be pivotal to BioMarin's evolution."

***Financial Highlights:***

- **Net Product Revenues** for the first quarter of 2021 decreased to $467.8 million, compared to $489.0 million for the same period in 2020. The decrease in Net Product Revenues was primarily attributed to the following:
    - Lower Kuvan product revenues primarily due to known generic competition in the U.S.; partially offset by
    - Higher Vimizim product revenues primarily due to timing of orders from Europe, Middle East and North Africa; and
    - Higher Palynziq product revenues primarily driven by a combination of revenue from more U.S. patients achieving maintenance dosing and new patients initiating therapy.

- **GAAP Net Incom**e decreased $64.0 million to $17.4 million for the first quarter of 2021 compared to $81.4 million for the same period in 2020. The decrease was primarily due to the absence of the gain on sale the Firdapse commercial assets totaling $59.5 million in the first quarter of 2020 and a decrease in gross profits due to the lower Kuvan product revenues due to generic competition in the U.S. This decrease was partially offset by lower selling, general and administrative (SG&A) expenses primarily due to a decrease in foreign currency exchange losses in the first quarter of 2021.
- **Non-GAAP Net Income** for the first quarter of 2021 decreased to $104.4 million compared to Non-GAAP Income of $116.5 million for the same period in 2020. The decline in Non-GAAP Income for the quarter, compared to the same period in 2020, was primarily attributed to lower gross profits, partially offset by lower SG&A expenses primarily due to a decrease in foreign currency exchange losses in the first quarter of 2021.

***Commercial Portfolio (Naglazyme, VIMIZIM, Brineura, Palynziq, Kuvan and Aldurazyme)***

- Naglazyme, VIMIZIM and Brineura maintained robust patient compliance in the quarter and continue to grow based on strong underlying demand.
    - Patients on commercial Naglazyme and Vimizim therapy both increased approximately 10% year-over-year.
    - Patients on commercial Brineura therapy increased by more than 30% year-over-year.
- Order timing for both Naglazyme and VIMIZIM are expected to be more concentrated in the first half of 2021 as compared to the second half of 2021.
- Palynziq top-line results in 2021 are expected to increase approximately 35% based on the mid-point of full-year 2021 guidance as compared to full-year 2020 results.
- Palynziq growth in European, Middle East and African regions (EMEA) has been impacted by ongoing challenges due to COVID-19 with Palynziq revenue from EMEA expected to increase when PKU clinics have more freedom to operate and start additional patients.
    - The number of U.S. Patients on commercial Palynziq therapy increased more than 20% year-over-year and is expected to continue to grow as U.S. PKU clinics re-open over the coming quarters.
- Loss of Kuvan U.S. market share, due to the introduction of generics following BioMarin's loss of U.S. market exclusivity, is consistent with expectations.
- Aldurazyme contributions were driven by the timing of product released and transfer of control to Genzyme, who is responsible for marketing Aldurazyme worldwide.

***Late-stage Regulatory Portfolio (Vosoritide and valoctocogene roxaparvovec)***

- In Europe, with respect to vosoritide for the treatment of achondroplasia, BioMarin is in the final stages of the review procedure ahead of the anticipated June 2021 Committee for Medicinal Products for Human Use (CHMP) opinion. Assuming a positive CHMP opinion, the European Commission could potentially grant marketing authorization for vosoritide in the third quarter of 2021.
- In Europe, with respect to valoctocogene roxaparvovec for the treatment of severe hemophilia A, based on positive pre-submission feedback in the current quarter, BioMarin reaffirms its plan to submit the Marketing Authorization Application with one-year results from the Phase 3 GENEr8-1 study to the European Medicines Agency (EMA) in June 2021.
- In the U.S., BioMarin provided the U.S. Food and Drug Administration (FDA) with the two-year results from the Phase 3 extension study with vosoritide to supplement the New Drug Application (NDA) already under review. As anticipated, the FDA designated this submission as a major amendment to the application, thus extending the Prescription Drug User Fee Act (PDUFA) target action date by three months to November 20, 2021 to provide time for a full review of the submission.
    - Also in the first quarter of 2021, the FDA pre-approval inspection of BioMarin's Novato facility for the manufacture of vosoritide drug substance was completed, representing another important milestone as vosoritide advances through the review process.
- During the current quarter, the FDA reiterated their recommendation that BioMarin submit two-year follow-up safety and efficacy data on all study participants from the GENEr8-1 study to support their benefit/risk assessment of valoctocogene roxaparvovec. BioMarin is targeting a Biologics License Application (BLA) submission in the second quarter of 2022 assuming favorable results, followed by an expected 6-month review procedure by the FDA.
    - The FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation to valoctocogene roxaparvovec. The RMAT designation is complementary to Breakthrough Therapy Designation, which BioMarin received in 2017.

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

- BMN 307: Dose escalation in PHEarless, the Phase 1/2 study of BMN 307 continues based on encouraging Phe lowering and safety profile observed in study participants who were treated with the lowest dose.
- BMN 255 for a subset of chronic renal disease: On January 11, 2021 BioMarin announced that it had filed an IND in 2020 for BMN 255, a small molecule for a subset of chronic renal disease. BMN 255 was driven by genetic discoveries for both mechanism and for identifying individuals for treatment.
- BMN 331 gene therapy product candidate for Hereditary Angioedema (HAE): IND-enabling studies are ongoing with BMN 331 for the treatment of HAE, BioMarin's third gene therapy candidate. BioMarin plans to leverage its broad expertise in developing gene therapies for severe hemophilia A and PKU to improve efficiencies in the development process of BMN 331. BioMarin is on track to file an IND for BMN 331 mid-year 2021.
- BMN 351 for Duchenne Muscular Dystrophy (DMD): IND-enabling studies are underway with BMN 351, an antisense oligonucleotide therapy that has demonstrated dystrophin expression levels of 30-50% of wild-type levels in the quadriceps in a DMD mouse model treated at 18.7 mg/kg/week for 13 weeks (measured 2 weeks following last administration). If results from the ongoing pre-clinical studies are supportive, BioMarin anticipates filing an IND for BMN 351 in the first half of 2022.
- DiNA-001 for MYBPC3 hypertrophic cardiomyopathy (HCM): Pre-clinical studies are underway with DiNA-001 following a collaboration announced in 2020 with DiNAQOR, a gene therapy platform company, to develop novel gene therapies to treat rare genetic cardiomyopathies. DiNAQOR received an undisclosed upfront payment and is eligible to receive development, regulatory and commercial milestones on product sales in addition to tiered royalties on worldwide sales.
- On April 28, 2021, BioMarin and the Allen Institute announced a collaboration to create new gene therapies aimed at rare genetic diseases of the central nervous system (CNS). The goal is to combine the Allen Institute's leadership in large-scale genomic science in the CNS therapeutic area with BioMarin's expertise in developing transformational gene therapies.

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

| Item | 2021 Guidance * (reaffirmed) | | |
|---|---|---|---|
| Total Revenues | $1,750 | to | $1,850 |
| Vimizim Net Product Revenues | $570 | to | $610 |
| Kuvan Net Product Revenues | $250 | to | $290 |
| Naglazyme Net Product Revenues | $365 | to | $395 |
| Palynziq Net Product Revenues | $210 | to | $250 |
| Brineura Net Product Revenues | $120 | to | $140 |
| Cost of Sales (% of Total Revenues) | 23% | to | 25% |
| Research and Development Expense | $645 | to | $695 |
| Selling, General and Administrative Expense | $725 | to | $775 |
| GAAP Net Loss | ($130) | to | ($80) |
| Non-GAAP Income [(1)] | $170 | to | $220 |

*2021 Guidance takes into consideration ongoing expected impact from the COVID-19 pandemic in 2021 assuming consistent trends experienced during 2020.

(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 page11 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 first quarter and full-year 2021 financial results today, Thursday, April 29, 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: 2396463 | Conference ID: 2396463 |

**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 BioMarin's clinical development and commercial prospects, including announcements of data from clinical studies and trials; the clinical development and commercialization of BioMarin's product candidates and commercial products, including (i) BioMarin's plan to submit complete one-year Phase 3 data for valoctocogene roxaparvovec to the EMA in June 2021, (ii) BioMarin's plan to re-submit its MAA for valoctocogene roxaparvovec to the EMA in June 2021, (iii) 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, (iv) that the CHMP opinion for vosoritide is expected in Europe in June 2021, (v) the extended PDUFA target action date with respect to vosoritide of November 20, 2021, (vi) BioMarin's anticipated IND submission for BMN 351 in the first half of 2022, and (vii) BioMarin and Allen Institute collaborating to create new gene therapies; the potential approval and commercialization of BioMarin's product candidates, including vosoritide for the treatment of achondroplasia and valoctocogene roxaparvovec for the treatment of severe hemophilia A, including timing of such approval decisions; and the expected benefits and availability of BioMarin's product candidates, including that vosoritide would be the first potential therapeutic option for children with achondroplasia; and potential growth opportunities and trends, including (i) that vosoritide would set the stage for the Company's next significant stage of growth if approved and (ii) growth expectations regarding Palynziq.

These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: BioMarin's success in the commercialization of its commercial products, including BioMarin's projected impact of the COVID-19 pandemic on its global revenue sources, including due to demand interruptions such as missed patient infusions and delayed treatment starts for new

patients; results and timing of current and planned preclinical studies and clinical trials, as well as the potential impact of the COVID-19 pandemic on (i) BioMarin's ability to continue such preclinical studies and clinical trials and (ii) the timing of such preclinical studies and clinical trials, and the release of data from those trials; BioMarin's ability to successfully manufacture its commercial products and product candidates; the content and timing of decisions by the FDA, the European Commission and other regulatory authorities concerning each of the described products and product candidates, including the potential impact of the COVID-19 pandemic on the regulatory authorities' abilities to issue such decisions and the timing of such decisions; the market for each of these products; actual sales of BioMarin's commercial products and the impact that the COVID-19 pandemic may have on such sales; the introduction of generic versions of BioMarin's commercial products, in particular generic versions of Kuvan; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission (SEC), including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's Annual Report on Form 10-K for the year ended December 31, 2020 as such factors may be updated by any subsequent reports. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise.



**BIOMARIN PHARMACEUTICAL INC.**

**CONDENSED CONSOLIDATED BALANCE SHEETS**

**March 31, 2021 and December 31, 2020**

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

| | March 31, 2021 | December 31, 2020[(1)] |
|---|---|---|
| **ASSETS** | (unaudited) | |
| Current assets: | | |
| Cash and cash equivalents | $ 667,313 | $ 649,158 |
| Short-term investments | 420,178 | 416,228 |
| Accounts receivable, net | 396,097 | 448,351 |
| Inventory | 713,929 | 698,548 |
| Other current assets | 91,249 | 129,934 |
| Total current assets | 2,288,766 | 2,342,219 |
| Noncurrent assets: | | |
| Long-term investments | 321,127 | 285,473 |
| Property, plant and equipment, net | 1,022,474 | 1,032,471 |
| Intangible assets, net | 405,903 | 417,271 |
| Goodwill | 196,199 | 196,199 |
| Deferred tax assets | 1,429,386 | 1,432,150 |
| Other assets | 141,198 | 142,237 |
| Total assets | | |

| | | |
|---|---|---|
| | $ 5,805,053 | $ 5,848,020 |
| **LIABILITIES AND STOCKHOLDERS' EQUITY** | | |
| Current liabilities: | | |
| Accounts payable and accrued liabilities | $ 399,225 | $ 492,548 |
| Short-term contingent consideration | 30,810 | — |
| Total current liabilities | 430,035 | 492,548 |
| Noncurrent liabilities: | | |
| Long-term convertible debt, net | 1,076,127 | 1,075,145 |
| Long-term contingent consideration | 29,153 | 60,130 |
| Other long-term liabilities | 107,728 | 114,195 |
| Total liabilities | $ 1,643,043 | $ 1,742,018 |
| Stockholders' equity: | | |
| Common stock, $0.001 par value: 500,000,000 shares authorized; 182,670,614 and 181,740,999 shares issued and outstanding, respectively. | 183 | 182 |
| Additional paid-in capital | 5,010,619 | 4,993,407 |
| Company common stock held by Nonqualified Deferred Compensation Plan | (9,558) | (9,839) |
| Accumulated other comprehensive income (loss) | 5,004 | (16,139) |
| Accumulated deficit | (844,238) | (861,609) |
| Total stockholders' equity | 4,162,010 | 4,106,002 |
| Total liabilities and stockholders' equity | $ 5,805,053 | $ 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 Months Ended March 31, 2021 and 2020**

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

| | Three Months Ended March 31, | |
|---|---|---|
| | **2021** | **2020** |
| | (unaudited) | (unaudited) |
| **REVENUES:** | | |
| Net product revenues | $ 467,769 | $ 489,043 |
| Royalty and other revenues | 18,261 | 13,026 |
| Total net revenues | 486,030 | 502,069 |
| **OPERATING EXPENSES:** | | |

| | | |
|---|---|---|
| Cost of sales | 120,166 | 111,374 |
| Research and development | 148,725 | 142,257 |
| Selling, general and administrative | 174,318 | 187,295 |
| Intangible asset amortization and contingent consideration | 17,735 | 15,677 |
| Gain on sale of nonfinancial assets | — | (59,495) |
| Total operating expenses | 460,944 | 397,108 |
| **INCOME FROM OPERATIONS** | 25,086 | 104,961 |
| Equity in the loss of BioMarin/Genzyme LLC | (1,351) | (77) |
| Interest income | 2,439 | 5,244 |
| Interest expense | (3,804) | (6,915) |
| Other income, net | 858 | (1,861) |
| **INCOME BEFORE INCOME TAXES** | 23,228 | 101,352 |
| Provision for income taxes | 5,857 | 19,971 |
| **NET INCOME** | $ 17,371 | $ 81,381 |
| **NET INCOME PER SHARE, BASIC** | $ 0.10 | $ 0.45 |
| **NET INCOME PER SHARE, DILUTED** | $ 0.09 | $ 0.44 |
| Weighted average common shares outstanding, basic | 181,772 | 179,898 |
| Weighted average common shares outstanding, diluted | 184,365 | 187,163 |

**BIOMARIN PHARMACEUTICAL INC.**

**CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS**

**Three Months Ended March 31, 2021 and 2020**

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

| | Three Months Ended March 31, | |
|---|---|---|
| | **2021** | **2020** |
| | (unaudited) | (unaudited) |
| **CASH FLOWS FROM OPERATING ACTIVITIES:** | | |
| Net income | $ 17,371 | $ 81,381 |
| Adjustments to reconcile net income to net cash used in operating activities: | | |
| Depreciation and amortization | 27,983 | 25,964 |
| Non-cash interest expense | 1,043 | 4,618 |
| Amortization of premium (accretion of discount) on investments | 673 | 60 |
| Stock-based compensation | 49,503 | 46,994 |
| Gain on sale of nonfinancial assets | — | (59,495) |
| Deferred income taxes | 3,335 | 10,603 |

| | | |
|---|---|---|
| Unrealized foreign exchange loss | 3,950 | 9,400 |
| Non-cash changes in the fair value of contingent consideration | 2,255 | (4) |
| Other | (871) | (383) |
| Changes in operating assets and liabilities: | | |
| Accounts receivable, net | 40,294 | (31,898) |
| Inventory | (6,425) | (20,706) |
| Other current assets | 42,784 | 8,302 |
| Other assets | 1,617 | (441) |
| Accounts payable and accrued liabilities | (72,304) | (94,733) |
| Other long-term liabilities | 2,304 | 5,144 |
| Net cash provided by (used in) operating activities | 113,512 | (15,194) |
| **CASH FLOWS FROM INVESTING ACTIVITIES:** | | |
| Purchases of property, plant and equipment | (25,507) | (40,554) |
| Maturities and sales of investments | 194,637 | 94,701 |
| Purchases of available-for-sale securities | (237,171) | (40,104) |
| Proceeds from sale of nonfinancial assets | — | 67,159 |
| Purchase of intangible assets | (2,747) | (3,463) |
| Other | — | (335) |
| Net cash provided by (used in) investing activities | (70,788) | 77,404 |
| **CASH FLOWS FROM FINANCING ACTIVITIES:** | | |
| Proceeds from exercises of awards under equity incentive plans | 5,817 | 10,116 |
| Taxes paid related to net share settlement of equity awards | (29,097) | (28,844) |
| Principal repayments of financing leases | (1,084) | (943) |
| Net cash used in financing activities | (24,364) | (19,671) |
| Effect of exchange rate changes on cash | (205) | (3,353) |
| **NET INCREASE IN CASH AND CASH EQUIVALENTS** | $ 18,155 | $ 39,186 |
| Cash and cash equivalents: | | |
| Beginning of period | $ 649,158 | $ 437,446 |
| End of period | $ 667,313 | $ 476,632 |

The following table presents Net Product Revenues by Product:

**Net Product Revenues by Product**

**(In millions of U.S. dollars)**

| | Three Months Ended March 31, | | |
|---|---|---|---|
| | **2021** | **2020** | **% Change** |

| | (unaudited) | (unaudited) | |
|---|---|---|---|
| PKU franchise | $ 124.8 | $ 156.6 | (20) % |
| Vimizim | 158.4 | 137.2 | 15 % |
| Naglazyme | 107.3 | 114.3 | (6) % |
| Brineura | 27.3 | 24.0 | 14 % |
| Firdapse [1] | — | 1.2 | (100) % |
| Net Product Revenues Marketed by BioMarin | $ 417.8 | $ 433.3 | |
| Aldurazyme Net Product Revenues Marketed by Genzyme | 50.0 | 55.7 | (10) % |
| Total Net Product Revenues | $ 467.8 | $ 489.0 | |

(1) In January 2020, BioMarin divested the Firdapse assets to a third party in a sale transaction. The sale is reflected in the Company's consolidated financial statements for the three months ending March 31, 2020; and as a result of the transaction BioMarin no longer generates Net Product Revenues from Firdapse.

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

**Net Product Revenues by Product for the PKU Franchise**

**(In millions of U.S. dollars)**

| | Three Months Ended March 31, | | |
|---|---|---|---|
| | **2021** | **2020** | **% Change** |
| | (unaudited) | (unaudited) | |
| Kuvan | $ 70.8 | $ 122.0 | (42) % |
| Palynziq | 54.0 | 34.6 | 56 % |
| Total PKU franchise | $ 124.8 | $ 156.6 | (20) % |

**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 March 31, | | Guidance Year Ending December 31, 2021 | | |
|---|---|---|---|---|---|
| | **2021** | **2020** | | | |
| **GAAP Net Income (Loss)** | **$ 17.4** | **$ 81.4** | **$ (130.0)** | **—** | **$ (80.0)** |
| Interest expense, net | 1.4 | 1.7 | 11.0 | — | 11.0 |
| Provision for income taxes | 5.9 | 20.0 | (16.0) | — | (16.0) |
| Depreciation expense | 12.5 | 10.3 | 48.0 | — | 48.0 |
| Amortization expense | 15.4 | 15.7 | 62.0 | — | 62.0 |
| Stock-based compensation expense | 49.5 | 46.9 | 186.0 | — | 186.0 |
| Contingent consideration expense | 2.3 | — | 9.0 | — | 9.0 |
| Gain on sale of nonfinancial assets | — | (59.5) | — | — | — |
| Non-GAAP Income | $ 104.4 | $ 116.5 | $ 170.0 | — | $ 220.0 |

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 In**

**(In millions of U.S. dollars)**

| | (unaudited) | | | |
|---|---|---|---|---|
| | Three months en | | | |
| | 2021 | | | |
| | | Adjustments | | |
| | GAAP Reported | Interest, Taxes, Depreciation and Amortization | Stock-Based Compensation, Contingent Consideration and Other Adjustments | Non-GAAP |
| Cost of sales | **$ 120.2** | $ — | $ (6.5) | $ 113.7 |
| Research and development | **148.7** | (7.5) | (17.5) | 123.7 |
| Selling, general and administrative | **174.3** | (5.0) | (25.5) | 143.8 |
| Intangible asset amortization and contingent consideration | **17.7** | (15.4) | (2.3) | — |
| Gain on sale of nonfinancial assets | **0.0** | — | — | — |
| Interest expense, net | **(1.4)** | 1.4 | — | — |
| Provision for income taxes | **5.9** | (5.9) | — | — |
| GAAP Net Income / Non-GAAP Income | **$ 17.4** | $ 35.2 | $ 51.8 | $ 104.4 |


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