BioMarin Announces First Quarter 2012 Financial Results

Financial Highlights ($ in millions, except per share data, unaudited) Item 1Q 2012 1Q 2011 Comparison Total BioMarin Revenue $116.6 6.6% increase Total Net Product Revenue $116.2 6.6% increase Naglazyme Net Product Revenue $68.6 13.2% increase Aldurazyme BioMarin Net Product Revenue* $12.0 $18.7 Kuvan Net Product Revenue $32.0 19.9% increase Firdapse Net Product Revenue $3.6 16.1% increase GAAP Net Loss $(24.0) $(4.4) GAAP Net Loss per share $(0.21) (basic and diluted) $(0.04) (basic and diluted) Non-GAAP Adjusted EBITDA Income (Loss) $(0.1) $17.3 * Net product transfer revenue had a negative $6.4 million impact on net Aldurazyme revenue to BioMarin in the first quarter of 2012 and a positive $1.8 million impact on net Aldurazyme revenue to BioMarin in the first quarter of 2011. Net product transfer revenue is expected to be neutral or positive in the second quarter of 2012 and positive in the second half of 2012.

NOVATO, Calif., April 26, 2012 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced financial results for the first quarter of 2012. GAAP net loss was $24.0 million ($0.21 per diluted share) for the first quarter of 2012, compared to GAAP net loss of $4.4 million ($0.04 per diluted share) for the first quarter of 2011. Non-GAAP adjusted EBITDA was a loss of $0.1 million for the first quarter of 2012, compared to non-GAAP adjusted EBITDA of $17.3 million for the first quarter of 2011. Non-GAAP adjusted EBITDA excludes depreciation and amortization, contingent consideration expense, interest income and expense, income taxes, stock compensation expense and material non-recurring items. The reconciliation of the non-GAAP measures to the comparable GAAP measure is detailed in the table provided near the end of the press release.

As of March 31, 2012, BioMarin had cash, cash equivalents and short and long-term investments totaling $287.7 million, as compared to $289.5 million on December 31, 2011.

"Our commercial portfolio is off to a strong start in 2012, as we surpassed $100 million in quarterly revenue for BioMarin marketed products for the first time. This growth was driven by Naglazyme net product revenue which was up 13 percent year over year and Kuvan net product revenue which was up 20 percent year over year. In early January, we booked an order from Brazil that was delayed from the fourth quarter of 2011, but even without that order, the first quarter of 2012 would have been our strongest Naglazyme quarter since launch," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "The successful and timely execution of our R&D pipeline remains our top priority as we look forward to five clinical program readouts in the second half of the year. Enrollment for the GALNS pivotal Phase 3 trial was completed in early March with 176 patients in 31 sites and 17 countries, more than the targeted 162 patients due to an overwhelming response from the MPS IVA community. In addition to the results of the Phase 3 GALNS trial, we also expect to have results from three Phase 2 programs - PEG-PAL for PKU, BMN-701 for Pompe disease, and BMN-673 for solid tumors - which could all potentially advance to Phase 3 trials in 2013."

Net Product Revenue (in millions)

Three Months Ended March 31, 2012 2011 $ Change % Change Naglazyme (1) $68.6 $60.6 $8.0 13.2% Kuvan (2) 32.0 26.7 5.3 19.9% Firdapse (3) 3.6 3.1 0.5 16.1%

(1) Changes in foreign currency rates, net of hedges, had a negative $0.2 million impact on Naglazyme sales in the first quarter of 2012. Naglazyme revenues experience quarterly fluctuations due to the timing of government ordering patterns in certain countries.

(2) The quantity of commercial tablets dispensed to patients in the U.S. increased 14.1 percent in the first quarter of 2011 compared to the first quarter of 2011.

(3) A product for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS) which was launched in the EU in April 2010.

Three Months Ended March 31, 2012 2011 $ Change % Change Aldurazyme revenue reported by Genzyme (4) $45.9 $42.8 $3.1 7.2% Royalties due from Genzyme 18.4 16.9 1.5 Incremental product transfer revenues (5) (6.4) 1.8 (8.2) Total Aldurazyme net product revenues $12.0 $18.7 $(6.7)

(4) The total number of Aldurazyme patients increased 9.1 percent in the first quarter of 2012 as compared to the first quarter of 2011.

(5) To the extent units shipped to third party customers by Genzyme exceeded BioMarin inventory transfers to Genzyme, BioMarin will record a decrease in net product revenue from the royalty payable to BioMarin for the amount of previously recognized product transfer revenue. If BioMarin inventory transfers exceed units shipped to third party customers by Genzyme, BioMarin will record incremental net product transfer revenue for the period.

2012 Guidance Revenue Guidance ($ in millions) Item 2012 Guidance Previous 2012 Guidance Total BioMarin Revenues $475 to $510 $465 to $510 Total Net Product Revenues $470 to $505 $460 to $505 Naglazyme Net Product Revenue $250 to $265 $240 to $265 Kuvan Net Product Revenue Unchanged $126 to $136 Aldurazyme Net Product Revenue to BioMarin Unchanged $81 to $87 Firdapse Net Product Revenue Unchanged $13 to $17 Selected Income Statement Guidance ($ in millions) Item 2012 Guidance Previous 2012 Guidance Cost of Sales (% of Total Revenue) Unchanged 17% to 18% Selling, General and Admin. Expense Unchanged $195 to $205 Research and Development Expense $265 to $275* $255 to $265 Amortization and Contingent Consideration Unchanged $19 Income Tax Expense Unchanged $ - GAAP Net (Loss) Unchanged $(92) to $(82) Stock Compensation Expense Unchanged $46 Non-GAAP Adjusted EBITDA Unchanged $15 to $25 Non GAAP Net Cash Flow (Usage) Unchanged $(30) to $(40) Cash Balance** Unchanged $250 to $260 * Approximately $52 million of the $265 million to $275 million in 2012 is for the production of drug supply for clinical studies. In the first quarter of 2012, R&D expenses were higher due to increased GALNS clinical and manufacturing activities. Additionally, R&D expenses were higher in the first quarter due to Phase 2 studies for PEG-PAL, BMN-701 and BMN-673. ** Cash balance includes cash, cash equivalents and short and long term investments

Anticipated Upcoming Milestones

3Q 2012: Results for Phase II trial for PEG-PAL for PKU 3Q 2012: Results for Phase I trial for BMN-111 for achondroplasia in healthy volunteers 2H 2012: Results for Phase I/II trial for BMN-673 for solid tumors 4Q 2012: Results for Phase III trial for GALNS for MPS IVA 4Q 2012: Results for Phase I/II trial for BMN-701 for Pompe disease 4Q 2012/1Q 2013: Initiation of Phase II trial for BMN-111 for achondroplasia in patients 1Q 2013: Market authorization application filings for GALNS for MPS IVA 1Q 2013: Results for Phase I trial for BMN-673 for hematological malignancies 1Q 2013: Potential initiation of Phase III trial for PEG-PAL for PKU 1Q 2013: IND filing for BMN-190 for LINCL (Batten disease) Mid-2013: Results for PKU-016 Kuvan neurocognitive study 2H 2013: Potential initiation of Phase III trial for BMN-673 for solid tumors or hematological malignancies 4Q 2013: Potential initiation of Phase III trial for BMN-701 for Pompe disease 4Q 2013: Anticipated FDA approval of GALNS for MPS IVA

Research and Development Programs

BioMarin continues to make significant investments in research and development to ensure persistent growth for the company. The current pipeline includes programs in various stages of development that are focused on treating a range of serious unmet medical needs.

Advanced Clinical Programs

GALNS for MPS IVA: BioMarin completed the enrollment and randomization of 176 patients at 31 sites in 17 countries in the pivotal Phase III trial in early March. The company is on track to report top-line results in the fourth quarter of 2012 and expects to submit the first market authorization application filing in the first quarter of 2013. Additionally, the company has begun three ancillary studies: a clinical trial for patients under five years of age is currently enrolling, a study to more completely document the health benefits of GALNS is screening patients for enrollment and a study in patients with limited ambulation to begin enrollment imminently.

Mid-Stage Clinical Programs

PEG-PAL for PKU: In the ongoing Phase II study, the rate of discontinuation due to adverse events remains low and virtually all patients who are able to achieve a therapeutic dose have their blood Phe levels lowered to less than 600 µmol/L, the target of therapeutic efficacy. The company has initiated Part D of the Phase II trial to characterize the safety of a shorter weekly induction regimen prior to switching over to the daily maintenance dose. Results are expected in the third quarter of 2012, and if successful, the Phase III study is expected to start in the first quarter of 2013 after meetings with regulatory authorities.

Kuvan Outcomes Study: PKU-016, a randomized, placebo-controlled, 13-week Kuvan outcomes study is ongoing and is approximately two-thirds enrolled. Endpoints include clinically validated measures of neuropsychiatric symptoms and, if successful, may enable a label amendment. The company expects to report top-line results in mid-2013.

Early-Stage Clinical Programs

BMN-701 for Pompe Disease: The company has continued dosing patients in the 20 mg/kg cohort of the Phase I/II trial using the new material from the 12,000L process. BMN-701 has been generally well-tolerated with a safety profile consistent with other enzyme replacement therapies. Top-line results for the Phase I/II trial, including data in at least 15 patients dosed at 20 mg/kg, are expected in the fourth quarter of 2012.

BMN-673 (PARP inhibitor): BioMarin has two ongoing trials for BMN-673: a Phase I/II open-label trial of once daily, orally administered BMN-673 for advanced or recurrent solid tumors and a Phase I, two-arm, open-label, dose escalation study for BMN-673 for the treatment of patients with advanced hematological malignancies. Top-line results are expected in the second half of 2012 and the first quarter of 2013, respectively. Over 20 patients have been dosed in the solid tumor study, and the company has not yet determined the maximum tolerated dose.

BMN-111 for Achondroplasia: BMN-111 is an analog of C-type Natriuretic Peptide (CNP), a small cyclic peptide that is a positive regulator of bone growth. The company initiated the Phase I study in healthy volunteers in February 2012 and expects results by the third quarter of 2012.

Preclinical Programs

BMN-190 for LINCL (Batten disease): BioMarin expects to file the IND for BMN-190 in the first quarter of 2013.

Other early stage programs: BioMarin is working on multiple additional early development opportunities.

Non-GAAP Financial Information and Reconciliation

The above results for the three months ended March 31, 2012 and March 31, 2011 and financial guidance for the year ending December 31, 2012 are presented both as determined in accordance with GAAP and on a non-GAAP basis. As used in this release, non-GAAP income is based on GAAP Earnings Before Interest, Taxes, Depreciation and Amortization (EBITDA) adjusted to exclude non-cash stock compensation expense, contingent consideration expense and certain nonrecurring material items (Non-GAAP Adjusted EBITDA).

The following table presents the reconciliation of non-GAAP to GAAP financial metrics:

Reconciliation of GAAP Net Loss to Non-GAAP Adjusted EBITDA Income (Loss) (in millions) (unaudited)
Three Months Ended March 31, Year Ending December 31, 2012 Guidance NOTES 2012 2011 GAAP Net Loss $ (24.0) $ (4.4) $(92.0) -- $(82.0) Interest expense, net 1.4 1.4 6.0 Income tax expense -- 4.8 -- Depreciation 7.3 4.7 30.0 Amortization 9.3 0.9 16.0 EBITDA Income (Loss) (6.0) 7.4 (40.0) - (30.0) Stock-based compensation 11.1 10.4 46.0 Contingent consideration (1) (5.2) (0.5) 9.0 Non-GAAP Adjusted EBITDA Income (Loss) $ (0.1) $ 17.3 $15.0 -- $25.0 (1) Represents the changes in the fair value of contingent acquisition consideration payable for the period. The change in the current quarter reflects changes in estimated probabilities and timing of achieving certain developmental milestones.

BioMarin believes that this non-GAAP information is useful to investors, taken in conjunction with BioMarin's GAAP information because it provides additional information regarding the performance of BioMarin's core ongoing business, Naglazyme, Kuvan, Aldurazyme and Firdapse and development of its pipeline. By providing information about both the overall GAAP financial performance and the non-GAAP measures that focus on continuing operations, the company believes that the additional information enhances investors' overall understanding of the company's business and prospects for the future. Further, the company uses both the GAAP and the non-GAAP results and expectations internally for its operating, budgeting and financial planning purposes and uses the Non-GAAP Adjusted EBITDA methodology in establishing corporate goals for internal compensation programs.

Diluted Earnings Per Share Calculation

The following table presents the computation of GAAP diluted earnings per share:

GAAP Basic and Diluted Loss per Common Share Three Months Ended March 31, 2012 2011 (unaudited) (unaudited) Numerator GAAP Net loss, basic $ (23,972) $ (4,371) Gain on Company common stock held in the Nonqualified Deferred Compensation Plan - (156) GAAP Net loss, diluted $ (23,972) $ (4,527) Denominator (in thousands of common shares): Basic weighted-average shares outstanding 115,070 110,652 Effect of dilutuve securities: Common stock held in the Nonqualified Deferred Compensation Plan - 91 Fully diluted weighted-average shares 115,070 110,743 GAAP Basic loss per common share $ (0.21) $ (0.04) GAAP Diluted loss per common share $ (0.21) $ (0.04)

Conference Call Details

BioMarin will host a conference call and webcast to discuss first quarter 2012 financial results today, Thursday, April 26, at 5:00 p.m. ET. This event can be accessed on the investor section of the BioMarin website at www.BMRN.com.

Date: April 26, 2012 Time: 5:00 p.m. ET U.S. / Canada Dial -in Number: 866.783.2141 International Dial-in Number: 857.350.1600 Participant Code: 88398685 Replay Dial -in Number: 888.286.8010 Replay International Dial -in Number: 617.801.6888 Replay Code: 63970352

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises four approved products and multiple clinical and pre-clinical product candidates. Approved products include Naglazyme® (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; Kuvan® (sapropterin dihydrochloride) Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; and Firdapse™ (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Product candidates include GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase III clinical development for the treatment of MPS IVA, amifampridine phosphate (3,4-diaminopyridine phosphate), which is currently in Phase III clinical development for the treatment of LEMS in the U.S., PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase II clinical development for the treatment of PKU, BMN-701, a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), which is currently in Phase I/II clinical development for the treatment of Pompe disease, BMN-673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase I/II clinical development for the treatment of genetically-defined cancers, and BMN-111, a modified C-nutriuretic peptide, which is currently in Phase I clinical development for the treatment of achondroplasia. For additional information, please visit www.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

The BioMarin Pharmaceutical Inc. logo is available at http://www.globenewswire.com/newsroom/prs/?pkgid=11419

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin

Pharmaceutical Inc., including, without limitation, statements about: the expectations of revenue and sales related to Naglazyme, Kuvan, Firdapse, and Aldurazyme; the financial performance of the BioMarin as a whole; the timing of BioMarin's clinical trials of GALNS, Firdapse, PEG-PAL, BMN-673, BMN-701, BMN-111 and other product candidates; the continued clinical development and commercialization of Aldurazyme, Naglazyme, Kuvan, Firdapse, and its product candidates; and actions by regulatory authorities. 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: our success in the continued commercialization of Naglazyme, Kuvan, and Firdapse; Genzyme Corporation's success in continuing the commercialization of Aldurazyme; results and timing of current and planned preclinical studies and clinical trials, particularly with respect to GALNS, Firdapse, PEG-PAL, BMN-673, BMN-701 and BMN-111; our ability to successfully manufacture our products and product candidates; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning each of the described products and product candidates; the market for each of these products and particularly Aldurazyme, Naglazyme, Kuvan and Firdapse; actual sales of Aldurazyme, Naglazyme Kuvan and Firdapse; Merck Serono's activities related to Kuvan; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's 2011 Annual Report on Form 10-K, and the factors contained in BioMarin's reports on Form 10-Q. 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, 2012 and December 31, 2011 (In thousands of U.S. dollars, except share and per share amounts) March 31, December 31, 2012 2011(1) ASSETS (unaudited) Current assets: Cash and cash equivalents $82,586 $46,272 Short-term investments 150,393 148,820 Accounts receivable, net (allowance for doubtful accounts: $471 and $513 , respectively) 105,828 104,839 Inventory 124,064 130,118 Other current assets 50,519 39,753 Total current assets 513,390 469,802 Investment in BioMarin/Genzyme LLC 1,082 559 Long-term investments 54,751 94,385 Property, plant and equipment, net 264,317 268,971 Intangible assets, net 170,914 180,277 Goodwill 51,543 51,543 Long-term deferred tax assets 221,239 222,649 Other assets 19,849 15,495 Total assets $1,297,085 $1,303,681 LIABILITIES AND STOCKHOLDERS' EQUITY Current liabilities: Accounts payable and accrued liabilities $96,359 $94,125 Convertible debt 23,455 - Total current liabilities 119,814 94,125 Long-term convertible debt 324,872 348,329 Other long-term liabilities 80,449 88,179 Total liabilities 525,135 530,633 Stockholders' equity: Common stock, $0.001 par value: 250,000,000 shares authorized at March 31, 2012 and December 31, 2011: 115,681,825 and 114,789,732 shares issued and outstanding at March 31, 2012 and December 31, 2011, respectively. 116 115 Additional paid-in capital 1,221,933 1,197,082 Company common stock held by Nonqualified Deferred Compensation Plan (3,538) (3,935) Accumulated other comprehensive income 2,512 4,887 Accumulated deficit (449,073) (425,101) Total stockholders' equity 771,950 773,048 Total liabilities and stockholders' equity $1,297,085 $1,303,681 (1) December 31, 2011 balances were derived from the audited consolidated financial statements.

BIOMARIN PHARMACEUTICAL INC. CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS Three Months Ended March 31, 2012 and 2011 (In thousands of U.S. dollars, except per share amounts) (Unaudited) 2012 2011 REVENUES: Net product revenues $116,239 $109,076 Collaborative agreement revenues 96 125 Royalty and license revenues 314 255 Total revenues 116,649 109,456 OPERATING EXPENSES: Cost of sales (excludes amortization of certain acquired intangible assets) 17,105 20,796 Research and development 73,834 45,017 Selling, general and administrative 45,248 41,037 Intangible asset amortization and contingent consideration 2,328 312 Total operating expenses 138,515 107,162 INCOME (LOSS) FROM OPERATIONS (21,866) 2,294 Equity in the loss of BioMarin/Genzyme LLC (734) (542) Interest income 505 782 Interest expense (1,947) (2,163) Other income and (expense) 36 22 INCOME (LOSS) BEFORE INCOME TAXES (24,006) 393 Provision for (benefit from) income taxes (34) 4,764 NET LOSS $(23,972) $(4,371) NET LOSS PER SHARE, BASIC AND DILUTED $(0.21) $(0.04) Weighted average common shares outstanding, basic 115,070 110,652 Weighted average common shares outstanding, diluted 115,070 110,743 COMPREHENSIVE LOSS $(26,347) $(10,346)

STOCK-BASED COMPENSATION EXPENSE Total stock-based compensation expense included in the Condensed Consolidated Statements of Comprehensive Loss is as follows: Three Months Ended March 31, 2012 2011 (unaudited) (unaudited) Cost of sales $873 $1,402 Research and development 4,695 3,674 Selling, general and administrative 5,566 5,304 $11,134 $10,380

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BioMarin Pharmaceutical Inc.
