# BioMarin Announces Fourth Quarter and Full Year 2012 Financial Results

**Total Revenue in 2012 Exceeds $500 Million**
**PEG-PAL End of Phase 2 Meeting Completed, Phase 3 to Begin Next Quarter**
**Conference Call and Webcast to Be Held Today at 5:00 p.m. ET**

**Financial Highlights ($ in millions, except per share data, unaudited)**

| | FY 2012 | FY 2011 | Percent Change |
|---|---|---|---|
| Total BioMarin Revenue | $ 500.7 | $ 441.4 | 13.4% |
| Total Net Product Revenue | 496.5 | 437.6 | 13.5% |
| Naglayzme Net Product Revenue | 257.0 | 224.9 | 14.3% |
| Aldurazyme BioMarin Net Product Revenue | 82.2 | 82.8 | -0.7% |
| Kuvan Net Product Revenue | 143.1 | 116.8 | 22.5% |
| Firdapse Net Product Revenue | 14.2 | 13.2 | 7.6% |
| GAAP Net Loss | (114.3) | (53.8) | |
| GAAP Net Loss per Share (basic and diluted) | $ (0.95) | $ (0.48) | |
| Non-GAAP Adjusted EBITDA Income (Loss) | $ (11.6) | $ 34.5 | |
| Cash, cash equivalents and short and long-term investments* | $ 566.7 | $ 289.5 | 95.8% |

* The cash balance at the end of 2012 includes net proceeds of $235.5 million from the public offering in June 2012.

SAN RAFAEL, Calif., Feb. 21, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced financial results for the fourth quarter and full year 2012. GAAP net loss was $53.0 million ($0.43 per share) for the fourth quarter of 2012, compared to GAAP net loss of $26.7 million ($0.23 per share) for the fourth quarter of 2011. Non-GAAP adjusted EBITDA was a loss of $15.5 million for the fourth quarter of 2012, compared to non-GAAP adjusted EBITDA loss of $3.0 million for the fourth quarter of 2011. The increased GAAP net loss and the reduced non-GAAP adjusted EBITDA for the fourth quarter of 2012 compared to the fourth quarter of 2011 was primarily due to increased net product revenue offset by a larger increase in research and development expenses.

GAAP net loss for the year ended December 31, 2012 was $114.3 million ($0.95 per share), compared to GAAP net loss of $53.8 million ($0.48 per share) for the year ended December 31, 2011. Non-GAAP adjusted EBITDA was a loss of $11.6 million for the year ended December 31, 2012, compared to non-GAAP adjusted EBITDA of $34.5 million for the year ended December 31, 2011. The increased GAAP net loss and the reduced non-GAAP adjusted EBITDA for the year ended December 31, 2012 compared to the year ended December 31, 2011 was primarily due to increased research and development expenses and increased selling, general and administrative expenses partially offset by increased net product revenue.

As of December 31, 2012, BioMarin had cash, cash equivalents and short and long-term investments totaling $566.7 million, as compared to $533.2 million on September 30, 2012.

"2012 was a milestone year for BioMarin. Our growing commercial portfolio helped us surpass $500 million in total revenue and the pipeline continued to advance, culminating in positive results for the pivotal Phase 3 study for Vimizim at the end of the year," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We are poised for additional clinical milestones in the first half of 2013 with key data readouts from BMN-701 for Pompe disease and BMN-673, our PARP inhibitor. We also expect to have our first regulatory approval for Vimizim by the end of 2013, which we believe will propel the company into its next stage of growth."

**Net Product Revenue (in millions)**

| | Three Months Ended December 31, | | | | Twelve Months Ended December 31, | | | |
|---|---|---|---|---|---|---|---|---|
| | 2012 | 2011 | $ Change | % Change | 2012 | 2011 | $ Change | % Change |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Naglazyme [1] | $ 63.0 | $ 48.1 | $ 14.9 | 31.0% | $ 257.0 | $ 224.9 | $ 32.1 | 14.3% |
| Kuvan | 40.0 | 30.8 | 9.2 | 29.9% | 143.1 | 116.8 | 26.3 | 22.5% |
| Firdapse | 3.4 | 3.3 | 0.1 | 3.0% | 14.2 | 13.2 | 1.0 | 7.6% |

(1) Naglazyme revenues experience quarterly fluctuations due to the timing of government ordering patterns in certain countries.

| | Three Months Ended December 31, | | | | Twelve Months Ended December 31, | | | |
|---|---|---|---|---|---|---|---|---|
| | **2012** | **2011** | **$ Change** | **% Change** | **2012** | **2011** | **$ Change** | **% Change** |
| Aldurazyme revenue reported by Genzyme | $ 53.1 | $ 48.8 | $ 4.3 | 8.8% | $ 193.1 | $ 185.2 | $ 7.9 | 4.3% |
| Royalties due from Genzyme | 23.9 | 21.0 | 2.9 | | 80.4 | 74.2 | 6.2 | |
| Incremental product transfer revenues [2] | 0.7 | 2.8 | (2.1) | | 1.8 | 8.6 | (6.8) | |
| Total Aldurazyme net product revenues | $ 24.6 | $ 23.8 | $ 0.8 | | $ 82.2 | $ 82.8 | $ (0.6) | |

(2) To the extent units shipped to third party customers by Genzyme exceed 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.

**2013 Guidance**

| Revenue Guidance ($ in millions) | |
|---|---|
| Item | 2013 Guidance |
| Total BioMarin Revenues | $530 to $555 |
| Naglazyme Net Product Revenue | $265 to $285 |
| Kuvan Net Product Revenue | $155 to $170 |

| Selected Income Statement Guidance ($ in millions) | |
|---|---|
| Item | 2013 Guidance |
| Cost of Sales (% of Total Revenue) | 17% to 18% |
| Selling, General and Admin. Expense | $220 to $250 |
| Research and Development Expense* | $340 to $380 |
| GAAP Net Loss | $(195) to $(170) |
| Non-GAAP Adjusted EBITDA (loss) | $(75) to $(50) |
| Cash Balance** | Over $420 |

* Research and Development expense guidance includes expenses associated with the Zacharon acquisition and the University of College London license agreement

** Cash balance includes cash, cash equivalents and short and long term investments

**Anticipated Upcoming Milestones**

1Q 2013: File market authorization application for Vimizim (GALNS) for MPS IVA in the U.S.

1Q 2013: Results for Phase 1/2 trial for BMN-701 for Pompe disease

1Q 2013: Clinical trial application filing for BMN-190 for LINCL (Batten disease)

2Q 2013: File market authorization application for Vimizim (GALNS) for MPS IVA in the EU

2Q 2013: Initiation of Phase 3 trial for PEG-PAL for PKU

2Q 2013: Presentation on Phase 1/2 BMN-673 solid tumor data at ASCO meeting

Mid 2013: Initiation of Phase 2 trial for BMN-111 for achondroplasia

4Q 2013: Potential initiation of Phase 3 trial for BMN-673 for solid tumors

4Q 2013: Potential FDA approval of Vimizim for MPS IVA

4Q 2013: Potential initiation of Phase 2/3 trial, a key component of the pivotal program for BMN-701 for Pompe disease

**Research and Development Programs**

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

**Advanced Clinical Programs**

- **Vimizim for MPS IVA:** The company is on track to begin submitting market authorization application filings by the end of the first quarter of 2013. The three ancillary studies are ongoing: a clinical trial for patients under five years of age (fully enrolled), a cardiopulmonary study to more completely document the health benefits of Vimizim (fully enrolled), and a study in patients with limited ambulation (enrolling).

**Mid-Stage Clinical Programs**

- **PEG-PAL for PKU:** The company plans to initiate a pivotal Phase 3 study in the second quarter of 2013, following a recent end of Phase 2 meeting with the FDA. The Phase 3 design includes (1) an open-label study to evaluate safety and blood Phe levels in naïve patients and (2) a randomized controlled study of the Phase 2 extension study patients to evaluate blood Phe levels and neurocognitive endpoints. The FDA indicated the possibility for an accelerated approval based on demonstrating sustained reduction in Phe levels, though full approval may require demonstration of neurocognitive improvement.

- **Kuvan Outcomes Study:** The company recently reported top-line results of a randomized, placebo-controlled, 13-week Kuvan outcomes study. The primary endpoint of the study was an attention deficit hyperactivity rating scale (ADHD-RS), a commonly used test to evaluate symptoms of inattentiveness and hyperactivity. Kuvan improved the ADHD-RS ($p=0.085$), driven by a statistically significant change in the inattention component of the score ($p=0.036$). The company plans to discuss the submission of this data with the FDA for possible inclusion in the Kuvan label.

**Early-Stage Clinical Programs**

- **BMN-701 for Pompe Disease:** Top-line results for the Phase 1/2 trial and a program go/no go decision are expected at the end of the first quarter of 2013. The company recently determined that if it proceeds with development of BMN-701, it will utilize a new cell line. If the decision is made to continue development, the company will be prepared to start a Phase 2/3 trial, a key component of the pivotal program for BMN-701 with the new cell line in the fourth quarter of 2013.

- **BMN-673 (PARP inhibitor):** An update on the Phase 1/2 study in solid tumors is expected in the second quarter of 2013 at the ASCO Annual Meeting in June 2013. A Phase 3 trial in advanced or recurrent solid tumors could start enrolling by the end of 2013.

- **BMN-111 for Achondroplasia:** The company plans to initiate a Phase 2 trial in patients in mid 2013. The primary objective of the clinical proof of concept study in pediatric patients will be to evaluate the safety and tolerability of daily subcutaneous (sc) injections of BMN-111 administered for six months. Secondary

objectives of the study will be to assess changes in annualized growth velocity, changes in absolute growth and changes in body proportions. Other exploratory objectives will also be assessed.

- **BMN-190 for LINCL (Batten disease):** BioMarin expects to file for its first clinical study of BMN-190 in the first quarter of 2013. Pre-CTA/IND meetings have already been held with MHRA BfArM, and FDA. BioMarin expects to begin enrolling the study mid year 2013.

**Preclinical Programs**

- **Other early stage programs:** BioMarin is working on multiple additional early development opportunities, including two new lead optimization programs gained through the acquisition of Zacharon Pharmaceuticals: inhibition of heparan sulfate synthesis for MPS III and inhibition of ganglioside synthesis for diseases such as Tay Sachs and Sandhoff. The company also announced today the licensing of a Factor VIII gene therapy research program for hemophilia A from University College London and St. Jude Children's Research Hospital.

**Non-GAAP Financial Information and Reconciliation**

The results for the three months and year ended December 31, 2012 and December 31, 2011 are all determined in accordance with GAAP except for non-GAAP adjusted EBITDA which is determined on a non-GAAP basis. As used in this release, non-GAAP adjusted EBITDA is based on GAAP earnings before interest, taxes, depreciation and amortization (EBITDA) and further adjusted to also exclude certain non-cash stock compensation expense, non-cash contingent consideration expense and certain other 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 December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|---|
| | **NOTES** | **2012** | **2011** | **2012** | **2011** |
| **GAAP Net Loss** | | **$ (53.0)** | **$ (26.7)** | **$ (114.3)** | **$ (53.8)** |
| Interest expense, net | | 1.2 | 1.1 | 5.0 | 5.4 |
| Income tax expense (benefit) | | 2.9 | 3.6 | (3.9) | 10.2 |
| Depreciation | | 6.6 | 5.7 | 27.5 | 24.4 |
| Amortization | | 2.6 | 1.5 | 17.3 | 4.4 |
| **EBITDA (Loss)** | | **(39.7)** | **(14.8)** | **(68.4)** | **(9.4)** |
| Stock-based compensation | | 12.1 | 11.2 | 48.0 | 43.8 |
| Contingent consideration | (1) | 12.1 | 0.6 | 8.8 | (1.8) |
| Material non-recurring | | | | | |
| Convertible debt exchange | (2) | -- | -- | | 1.9 |
| **Non-GAAP Adjusted EBITDA Income (Loss)** | | **$ (15.5)** | **$ (3.0)** | **$ (11.6)** | **$ 34.5** |

(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.

(2) Represents debt conversion expense associated with the early conversion of a portion of our convertible debt in September 2011.

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.

**Conference Call Details**

BioMarin will host a conference call and webcast to discuss fourth quarter and full year 2012 financial results today, Thursday, February 21, at 5:00 p.m. ET. This event can be accessed on the investor section of the BioMarin website at www.BMRN.com.

Date: February 21, 2013
Time: 5:00 p.m. ET
U.S. / Canada Dial-in Number: 877.303.6313
International Dial-in Number: 631.813.4734
Conference ID: 49613469

Replay Dial-in Number: 855.859.2056
Replay International Dial-in Number: 404.537.3406
Conference ID: 49613469

**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 Vimizim (N-acetylgalactosamine 6-sulfatase), formally referred to as GALNS, which successfully completed Phase III clinical development for the treatment of MPS IVA, 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-natriuretic 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 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 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**
**December 31, 2012 and December 31, 2011**
**(In thousands of U.S. dollars, except share and per share amounts)**

| | December 31, 2012 | December 31, 2011(1) |
|---|---|---|
| **ASSETS** | (unaudited) | |
| Current assets: | | |
| Cash and cash equivalents | $ 180,527 | $ 46,272 |
| Short-term investments | 270,211 | 148,820 |
| Accounts receivable, net (allowance for doubtful accounts: $348 and $513, respectively) | 109,066 | 104,839 |
| Inventory | 128,695 | 130,118 |
| Current deferred tax assets | 29,454 | 21,115 |
| Other current assets | 25,509 | 18,638 |
| Total current assets | 743,462 | 469,802 |
| Noncurrent assets: | | |
| Investment in BioMarin/Genzyme LLC | 1,080 | 559 |
| Long-term investments | 115,993 | 94,385 |
| Property, plant and equipment, net | 284,473 | 268,971 |
| Intangible assets, net | 162,980 | 180,277 |
| Goodwill | 51,543 | 51,543 |
| Long-term deferred tax assets | 225,501 | 224,677 |
| Other assets | 16,611 | 15,495 |
| Total assets | $ 1,601,643 | $ 1,305,709 |
| **LIABILITIES AND STOCKHOLDERS' EQUITY** | | |
| Current liabilities: | | |
| Accounts payable and accrued liabilities | $ 147,068 | $ 94,125 |
| Convertible debt | 23,365 | -- |
| Total current liabilities | 170,433 | 94,125 |
| Noncurrent liabilities: | | |
| Long-term convertible debt | 324,859 | 348,329 |

| | | |
|---|---|---|
| Long-term contingent acquisition consideration payable | 30,618 | 33,059 |
| Long-term deferred tax liabilities | 33,296 | 37,155 |
| Other long-term liabilities | 26,674 | 19,993 |
| Total liabilities | 585,880 | 532,661 |
| Stockholders' equity: | | |
| Common stock, $0.001 par value: 250,000,000 shares authorized at December 31, 2011 and 2012; 125,809,162 and 114,789,732 shares issued and outstanding at December 31, 2012 and 2011, respectively. | 126 | 115 |
| Additional paid-in capital | 1,561,890 | 1,197,082 |
| Company common stock held by Nonqualified Deferred Compensation Plan | (6,603) | (3,935) |
| Accumulated other comprehensive income (loss) | (202) | 4,887 |
| Accumulated deficit | (539,448) | (425,101) |
| Total stockholders' equity | 1,015,763 | 773,048 |
| Total liabilities and stockholders' equity | $ 1,601,643 | $ 1,305,709 |

(1) December 31, 2011 balances were derived from the audited consolidated financial statements.

**BIOMARIN PHARMACEUTICAL INC.**

**CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS**
**Three and Twelve Months Ended December 31, 2012 and 2011**
**(In thousands of U.S. dollars, except per share amounts)**
**(Unaudited)**

| | Three Months Ended December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | **2012** | **2011** | **2012** | **2011** |
| **REVENUES:** | | | | |
| Net product revenues | $ 130,957 | $ 106,064 | $ 496,497 | $ 437,647 |
| Collaborative agreement revenues | 226 | 93 | 1,955 | 468 |
| Royalty and license revenues | 755 | 1,689 | 2,271 | 3,243 |
| Total revenues | 131,938 | 107,846 | 500,723 | 441,358 |
| **OPERATING EXPENSES:** | | | | |
| Cost of sales (excludes amortization of certain acquired intangible assets) | 26,532 | 21,519 | 91,830 | 84,023 |
| Research and development | 84,363 | 57,908 | 302,218 | 214,374 |
| Selling, general and administrative | 55,049 | 48,454 | 198,173 | 175,423 |
| Intangible asset amortization and contingent consideration | 12,898 | 1,400 | 18,717 | 1,428 |
| Total operating expenses | 178,842 | 129,281 | 610,938 | 475,248 |
| **LOSS FROM OPERATIONS** | (46,904) | (21,435) | (110,215) | (33,890) |
| Equity in the loss of BioMarin/Genzyme LLC | (253) | (609) | (1,221) | (2,426) |
| Interest income | 765 | 632 | 2,584 | 2,934 |
| Interest expense | (1,930) | (1,878) | (7,639) | (8,409) |
| Debt conversion expense | -- | -- | -- | (1,896) |
| Other income and (expense) | (1,772) | 174 | (1,787) | 60 |
| **LOSS BEFORE INCOME TAXES** | (50,094) | (23,116) | (118,278) | (43,627) |
| Provision for (benefit from) income taxes | 2,918 | 3,619 | (3,931) | 10,209 |
| **NET LOSS** | $ (53,012) | $ (26,735) | $ (114,347) | $ (53,836) |

| | | | | |
|---|---|---|---|---|
| **NET LOSS PER SHARE, BASIC AND DILUTED** | $ (0.43) | $ (0.23) | $ (0.95) | $ (0.48) |
| Weighted average common shares outstanding, basic and diluted | 124,575 | 114,415 | 120,271 | 112,122 |

**STOCK-BASED COMPENSATION EXPENSE**

Total stock-based compensation expense included in the Condensed Consolidated Statements of Operations is as follows:

| | **Three Months Ended December 31,** | | **Twelve Months Ended December 31,** | |
|---|---|---|---|---|
| | **2012** | **2011** | **2012** | **2011** |
| | **(unaudited)** | **(unaudited)** | **(unaudited)** | **(unaudited)** |
| Cost of sales | $ 1,355 | $ 1,307 | $ 4,890 | $ 5,171 |
| Research and development | 5,385 | 4,295 | 20,736 | 16,365 |
| Selling, general and administrative | 5,325 | 5,610 | 22,346 | 22,283 |
| | $ 12,065 | $ 11,212 | $ 47,972 | $ 43,819 |

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