BioMarin Announces First Quarter 2013 Financial Results

Total Revenue Continues Steady Growth of 10 Percent; Progress on All Aspects of the Business
Conference Call and Webcast to Be Held Today at 5:00 p.m. ET

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

	Q1 2013	Q1 2012	Percent Change
Total BioMarin Revenue	$ 127.9	$ 116.6	9.7%
Total Net Product Revenue	127.3	116.2	9.6%
Naglayzme Net Product Revenue	69.4	68.6	1.2%
Aldurazyme BioMarin Net Product Revenue	16.7	12.0	39.2%
Kuvan Net Product Revenue	37.6	32.0	17.5%
Firdapse Net Product Revenue	3.6	3.6	0.0%
GAAP Net Loss	(39.8)	(24.0)	
GAAP Net Loss per Share (basic and diluted)	$ (0.31)	$ (0.21)	
Non-GAAP Adjusted EBITDA Loss	$ (8.0)	$ (0.1)	
Cash, cash equivalents and short and long-term investments	$ 525.7	$ 287.7	82.7%

SAN RAFAEL, Calif., April 25, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced financial results for its first quarter ended March 31, 2013. GAAP net loss was $39.8 million ($0.31 per share) for the first quarter of 2013, compared to GAAP net loss of $24.0 million ($0.21 per share) for the first quarter of 2012. Non-GAAP adjusted EBITDA was a loss of $8.0 million for the first quarter of 2013, compared to non-GAAP adjusted EBITDA loss of $0.1 million for the first quarter of 2012. The increased GAAP net loss and the increased non-GAAP adjusted EBITDA loss for the first quarter of 2013 compared to the first quarter of 2012 was primarily due to increased net product revenue offset by a larger increase in research and development expenses.

As of March 31, 2013, BioMarin had cash, cash equivalents and short and long-term investments totaling $525.7 million, as compared to $566.7 million on December 31, 2012.

"In the first quarter, we continued to execute on our development goals as we head into another potentially transformative year for the company," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We made good progress on a number of important fronts including commercial, R&D, regulatory and business development. Highlights included positive results for the Phase 1/2 trial for BMN-701 for Pompe disease, submission of the BLA for Vimizim in the U.S. and the MAA for Vimizim in the EU, the acquisition of Zacharon Pharmaceuticals and the licensing of the Factor VIII program for Hemophilia A. We hit all of our stated milestones for the quarter and remain on track to meet our upcoming goals for the remainder of the year."

	Three Months Ended March 31,			
	2013	2012	$ Change	% Change
Naglazyme [(1)]	$ 69.4	68.6	$ 0.8	1.2%
Kuvan	37.6	32.0	5.6	17.5%
Firdapse	3.6	3.6	0.0	0.0%

(1) Naglazyme revenues experience quarterly fluctuations due to the timing of government ordering patterns in certain countries. There were no abnormal ordering patterns in the first quarter of 2013. In the first quarter of 2012, there was a delayed order from the fourth quarter of 2011. There was a 10.2 percent increase in Naglazyme revenue in the first quarter of 2013 over the fourth quarter of 2012 and an 11.0 percent increase in patients on therapy as compared to the first quarter of 2012, which is consistent with that of previous quarters.

	Three Months Ended March 31,

	2013	2012	$ Change	% Change
Aldurazyme revenue reported by Genzyme	$ 48.4	$ 45.9	$ 2.5	5.4%
Royalties due from Genzyme	19.3	18.4	0.9	
Incremental product transfer revenues [(2)]	(2.6)	(6.4)	3.8	
Total Aldurazyme net product revenues	$ 16.7	$ 12.0	$ 4.7	

(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.

BioMarin Reaffirms 2013 Full Year Financial 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 licensing of the Factor VIII program for Hemophilia A from the University College London and St. Jude Children's Research Hospital

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

Anticipated Upcoming Milestones

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
Mid 2013: Initiation of Phase 1/2 trial for BMN-190 for Batten disease
4Q 2013: Initiation of Phase 3 trial for BMN-673
4Q 2013: Potential FDA approval of Vimizim for MPS IVA
4Q 2013: Initiation of Phase 2/3 switching trial 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 submitted the BLA for Vimizim at the end of the first quarter of 2013 and the market authorization application filing in the EU earlier this week. Vimizim has received accelerated review status from the EMA.

Mid-Stage Clinical Programs

- **PEG-PAL for PKU:** The company plans to initiate a pivotal Phase 3 study in the second quarter of 2013. 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 and patients from the open label trial 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.

Early-Stage Clinical Programs

- **BMN-701 for Pompe Disease:** The company plans to initiate a Phase 2/3 switching trial by the end of 2013 in late-onset Pompe patients who have previously been treated with alglucosidase alfa. Subject to discussions with health authorities, the proposed study design is a single arm trial, with treatment at 20 mg/kg administered every other week for 24 weeks. The company intends to use the respiratory parameter MIP as the primary endpoint. Secondary objectives include MEP and six-minute walk test, as well as safety. The study will be conducted with full scale material from a revised manufacturing process, which has improved process robustness and increased productivity.
- **BMN-673 (PARP inhibitor):** An update on the Phase 1/2 study in solid tumors is expected at the ASCO Annual Meeting in June 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 filed a CTA in the first quarter of 2013 and expects to begin enrolling the study in mid 2013. Orphan drug designation has been granted in both the U.S. and EU.

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 has an ongoing 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 ended March 31, 2013 and March 31, 2012 and financial guidance for the year ending December 31, 2013 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 Loss
(in millions)
(unaudited)

		Three Months Ended March 31,		Year Ending December 31, 2013
	NOTES	2013	2012	Guidance

GAAP Net Loss		**$ (39.8)**	**$ (24.0)**	**$(195.0) - $(170.0)**
Interest expense, net		1.0	1.4	0.7
Benefit from Income taxes		(4.7)	--	(14.6)
Depreciation expense		6.1	7.3	25.0
Amortization expense		2.6	9.3	10.5
EBITDA Loss		**(34.8)**	**(6.0)**	**(173.4) - (148.4)**
Stock-based compensation expense		11.6	11.1	70.0
Contingent consideration expense	(1)	4.8	(5.2)	18.0
Material non-recurring:				
Debt conversion expense	(2)	10.4	--	10.4
Non-GAAP Adjusted EBITDA Loss		**$ (8.0)**	**$ (0.1)**	**$(75.0) - $(50.0)**

(1) Represents the expense 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 2017 convertible notes in March 2013.

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 first quarter 2013 financial results today, Thursday, April 25, 2013 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 25, 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: 29712683

Replay Dial-in Number: 855.859.2056
Replay International Dial-in Number: 404.537.3406
Conference ID: 29712683

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.

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; our expectations regarding the progress and 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; our expectations regarding the timing of our regulatory filings for our 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 risks that are discussed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, BioMarin's 2012 Annual Report on Form 10-K, and our periodic reports on Form 10-Q and Form 8-K. 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, 2013 and December 31, 2012
(In thousands of U.S. dollars, except share and per share amounts)

	March 31, 2013	December 31, 2012(1)
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 154,380	$ 180,527
Short-term investments	247,502	270,211
Accounts receivable, net (allowance for doubtful accounts: $376 and $348, respectively)	120,345	109,066
Inventory	135,822	128,695
Current deferred tax assets	29,474	29,454

Other current assets	35,501	25,509
Total current assets	723,024	743,462
Noncurrent assets:		
Investment in BioMarin/Genzyme LLC	679	1,080
Long-term investments	123,819	115,993
Property, plant and equipment, net	281,865	284,473
Intangible assets, net	172,016	162,980
Goodwill	54,975	51,543
Long-term deferred tax assets	226,757	225,501
Other assets	15,158	16,611
Total assets	$ 1,598,293	$ 1,601,643
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 144,008	$ 147,068
Convertible debt	--	23,365
Total current liabilities	144,008	170,433
Noncurrent liabilities:		
Long-term convertible debt	109,849	324,859
Long-term contingent acquisition consideration payable	27,224	30,618
Long-term deferred tax liabilities	37,521	33,296
Other long-term liabilities	29,539	26,674
Total liabilities	348,141	585,880
Stockholders' equity:		
Common stock, $0.001 par value: 250,000,000 shares authorized at March 31, 2013 and December 31, 2012; 138,873,207 and 125,809,162 shares issued and outstanding at March 31, 2013 and December 31, 2012, respectively.	139	126
Additional paid-in capital	1,833,831	1,561,890
Company common stock held by Nonqualified Deferred Compensation Plan	(5,715)	(6,603)
Accumulated other comprehensive income (loss)	1,155	(202)
Accumulated deficit	(579,258)	(539,448)
Total stockholders' equity	1,250,152	1,015,763
Total liabilities and stockholders' equity	$ 1,598,293	$ 1,601,643

(1) December 31, 2012 balances were derived from the audited consolidated financial statements.

BIOMARIN PHARMACEUTICAL INC.

CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
Three Months Ended March 31, 2013 and 2012
(In thousands of U.S. dollars, except per share amounts)
(Unaudited)

	2013	**2012**
REVENUES:		
Net product revenues	$ 127,344	$ 116,239
Collaborative agreement revenues	135	96
Royalty and license revenues	449	314

Total revenues	127,928	116,649
OPERATING EXPENSES:		
Cost of sales (excludes amortization of certain acquired intangible assets)	20,500	17,105
Research and development	83,743	73,834
Selling, general and administrative	51,050	45,248
Intangible asset amortization and contingent consideration	5,556	2,328
Total operating expenses	160,849	138,515
LOSS FROM OPERATIONS	(32,921)	(21,866)
Equity in the loss of BioMarin/Genzyme LLC	(401)	(734)
Interest income	718	505
Interest expense	(1,725)	(1,947)
Debt conversion expense	(10,420)	--
Other income	228	36
LOSS BEFORE INCOME TAXES	(44,521)	(24,006)
Provision for income taxes	(4,711)	(34)
NET LOSS	$ (39,810)	$ (23,972)
NET LOSS PER SHARE, BASIC AND DILUTED	$ (0.31)	$ (0.21)
Weighted average common shares outstanding, basic and diluted	127,969	115,070
COMPREHENSIVE LOSS	$ (38,452)	$ (26,347)

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,	
	2013 (unaudited)	**2012 (unaudited)**
Cost of sales	$ 1,044	$ 873
Research and development	5,324	4,695
Selling, general and administrative	5,197	5,566
	$ 11,565	$ 11,134

CONTACT: Investors:

Eugenia Shen

BioMarin Pharmaceutical Inc.

(415) 506-6570

Media:

Debra Charlesworth

BioMarin Pharmaceutical Inc.

(415) 455-7451

https://investors.biomarin.com/2013-04-25-BioMarin-Announces-First-Quarter-2013-Financial-Results