BioMarin Announces Second Quarter 2013 Financial Results

Total Revenue Growth Exceeds 10 Percent

Phase 3 Trial to Start in 3Q 2013

Conference Call and Webcast to be Held Today at 5:00 p.m. ET

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

	Q2 2013	Q2 2012	Percent Change
Total BioMarin Revenue	$ 136.8	$ 124.0	10.3%
Total BioMarin Revenue (excluding Aldurazyme Net Product Transfer Revenue) - non-GAAP	140.5	120.6	16.5%
Naglazyme Net Product Revenue	69.9	62.9	11.1%
Aldurazyme BioMarin Net Product Revenue	17.5	21.8	-19.7%
Aldurazyme Royalty Revenue (excluding Net Product Transfer Revenue) - non-GAAP	21.2	18.4	15.2%
Kuvan Net Product Revenue	40.9	34.7	17.9%
Firdapse Net Product Revenue	4.1	3.6	13.9%
Net Loss	(21.5)	(32.0)	
Net Loss per Share (basic)	$ (0.15)	$ (0.27)	
Net Loss per Share (diluted)	$ (0.16)	$ (0.27)	
Adjusted EBITDA Loss - non-GAAP	$ 0.0	$ (7.3)	
Cash, cash equivalents and short and long-term investments	$ 524.4	$ 524.6	0.0%

SAN RAFAEL, Calif., July 25, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced financial results for its second quarter ended June 30, 2013. GAAP net loss was $21.5 million ($0.16 per diluted share) for the second quarter of 2013, compared to GAAP net loss of $32.0 million ($0.27 per diluted share) for the second quarter of 2012. GAAP net loss for the six months ended June 30, 2013 was $61.3 million ($0.46 per

diluted share), as compared to GAAP net loss of $56.0 million ($0.48 per diluted share) for the six months ended June 30, 2012. Non-GAAP adjusted EBITDA was $0.0 million for the second quarter of 2013, compared to non-GAAP adjusted EBITDA loss of $7.3 million for the second quarter of 2012. Non-GAAP adjusted EBITDA was a loss of $8.0 million for the six months ended June 30, 2013, as compared to a loss of $7.2 million for the six months ended June 30, 2012. The decreased GAAP net loss and the decreased non-GAAP adjusted EBITDA loss for the second quarter of 2013 compared to the second quarter of 2012 was primarily due to higher total revenue and a smaller increase in operating expenses.

As of June 30, 2013, BioMarin had cash, cash equivalents and short and long-term investments totaling $ 524.4 million, as compared to $525.7 million on March 31, 2013.

"In the second quarter, we continued to execute on our development goals as we head into the second half of the year," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We made good progress in a number of important areas including commercial, R&D and regulatory. Highlights included initiation of the Phase 3 PEG-PAL trial for PKU, confirmation of priority review status for Vimizim in the U.S., validation of the MAA by the EMA, submission of the Vimizim MA in Brazil and a positive update of the ongoing BMN 673 trial for genetically-defined cancers at the ASCO annual meeting. We are pleased with the momentum of the BMN 673 program because of maturing and improving responses, completed positive regulatory discussions and earlier Phase 3 timelines than originally anticipated. We remain on track to meet our upcoming goals for the remainder of the year."

Net Product Revenue (in millions)

	Three Months Ended June 30,				Six Months Ended June 30,			
	2013	2012	$ Change	% Change	2013	2012	$ Change	% Change
Naglazyme [1]	$ 69.9	$ 62.9	$ 7.0	11.1%	$ 139.3	$ 131.5	$ 7.8	5.9%
Kuvan	40.9	34.7	6.2	17.9%	78.5	66.7	11.8	17.7%
Firdapse	4.1	3.6	0.5	13.9%	7.7	7.2	0.5	6.9%
Aldurazyme	17.5	21.8	(4.3)	-19.7%	34.2	33.8	0.4	1.2%
Net Product Revenues	132.4	123.0	9.4	7.6%	259.7	239.2	20.5	8.6%
Collaborative agreement revenues	0.9	0.4	0.5		1.0	0.6	0.4	
Royalty and license revenue	3.5	0.6	2.9		4.0	0.9	3.1	
Total BioMarin Revenues - GAAP	136.8	124.0	12.8	10.3%	264.7	240.7	24.0	10.0%
Less: Aldurazyme Net Product Transfer Revenues	(3.7)	3.4	(7.1)		(6.3)	(3.0)	(3.3)	
Total BioMarin Revenues (excluding Aldurazyme Net Product Transfer Revenue) - non-GAAP [2]	$ 140.5	$ 120.6	$ 19.9	16.5%	$ 271.0	$ 243.7	$ 27.3	11.2%

Reconciliation of Aldurazyme Revenue (in millions)

	Three Months Ended June 30,				Six Months Ended June 30,			
	2013	2012	$ Change	% Change	2013	2012	$ Change	% Change
Aldurazyme revenue reported by Genzyme	$53.6	$45.8	$7.8	17.0%	$102.1	$91.7	$ 10.4	11.3%
Aldurazyme Royalties due from Genzyme - Non-GAAP [2]	21.2	18.4	2.8		40.5	36.8	3.7	
Incremental product transfer revenues [3]	(3.7)	3.4	(7.1)		(6.3)	(3.0)	(3.3)	
Total Aldurazyme net product revenues - GAAP	$17.5	$21.8	$(4.3)		$34.2	$33.8	$0.4	

(1) Naglazyme revenues experience quarterly fluctuations due to the timing of government ordering

patterns in certain countries.

(2) 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 business. By providing information about both the GAAP and non-GAAP revenue measures, the company believes that the additional information enhances investors' overall understanding of the company's business and in particular allows for more consistent period to period evaluation of the revenue.

(3) 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 Full Year Financial Guidance

Revenue Guidance ($ in millions)

Item	2013 Guidance	Previous 2013 Guidance
Total BioMarin Revenues	$530 to $555	Unchanged
Naglazyme Net Product Revenue	$265 to $285	Unchanged
Kuvan Net Product Revenue	$155 to $170	Unchanged

Selected Income Statement Guidance ($ in millions)

Item	2013 Guidance	Previous 2013 Guidance
Cost of Sales (% of Total Revenue)	17% to 18%	Unchanged
Selling, General and Admin. Expense	$220 to $240	$220 to $250
Research and Development Expense	$340 to $380	Unchanged
GAAP Net Loss	$(185) to $(160)	$(195) to $(170)
Non-GAAP Adjusted EBITDA (loss)	$(65) to $(40)	$(75) to $(50)
Cash Balance*	Over $440	Over $420

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

Anticipated Upcoming Milestones

3Q 2013: Initiation of Phase 1/2 trial for BMN 190 for Batten disease

3Q 2013: Initiation of Phase 3 trial for BMN 673 for BRCA breast cancer
4Q 2013: Initiation of Phase 2/3 switching trial for BMN 701 for Pompe disease
4Q 2013: Potential CHMP opinion for Vimizim for MPA IVA
4Q 2013/1Q 2014: Initiation of Phase 1/2 trial for BMN 111 for achondroplasia
1Q 2014: PDUFA date for Vimizim for MPS IVA
1Q 2014: Potential launch of Vimizim for MPS IVA
4Q 2014: Top-line results for Phase 3 trial for PEG-PAL for PKU

Research and Development Programs

BioMarin continues to make significant progress in research and development to ensure a strong 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.

Programs Under Regulatory Review for Approval

- **Vimizim for MPS IVA:** During the second quarter, regulatory advancements were made worldwide. The FDA granted Vimizim priority review designation and assigned a PDUFA date of February 28, 2014. The EMA has validated the Vimizim MAA and has granted accelerated review status, which could lead to a CHMP opinion before year end. During the second quarter, the company also submitted the MA in Brazil. Ongoing production of bulk drug substance and drug product will allow for a global launch following approvals in multiple countries. Both the FDA and EMA have conducted or scheduled pre-approval inspections associated with the review of the marketing applications.

Advanced Clinical Programs

- **PEG-PAL for PKU:** The company initiated a pivotal Phase 3 study in the second quarter of 2013. The Phase 3 program 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, which is expected to be initiated in the third quarter of 2013. The company expects top-line results for the Phase 3 study in the fourth quarter of 2014.

- **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 and the respiratory parameter MIP as the primary endpoint. The company has completed a full scale production campaign using its new higher producing cell line and has verified the production yields. Characterization testing is currently ongoing, and it is expected that regulatory filings to support the introduction of this material into clinical studies will be submitted in the fourth quarter of 2013.

- **BMN 673 (PARP inhibitor):** As of July 24, the RECIST response rate from the ongoing trial is nine out of 18 breast cancer patients, or 50 percent, including one confirmed complete response. This response rate includes three additional confirmed responses since the last update at the ASCO Annual Meeting, and two new patients are yet to be confirmed. Four patients are ongoing with stable disease with potential for additional responses. All patients have been on treatment

for at least twelve weeks. The study is still ongoing, and the company will provide additional updates later this year, including data in ovarian cancer, Ewing's sarcoma and small cell lung cancer. BioMarin now expects to initiate a Phase 3 trial for BMN 673 in deleterious gBRCA mutation metastatic breast cancer in late third quarter of 2013, earlier than previously announced at the ASCO Annual Meeting in early June.

Early-Stage Clinical Programs

- **BMN 111 for Achondroplasia:** The company is modifying its clinical program based on a request from the Food and Drug Administration (FDA) for more clinical pharmacokinetic and safety data in children with the disease. BioMarin previously completed a Phase 1 study in adult healthy volunteers. Although the FDA acknowledges that the company has identified a range of doses that are relatively well-tolerated short—term, it has now requested additional PK and safety data in children with achondroplasia before proceeding to extended dosing in children. The FDA has placed the BMN 111 program on partial clinical hold. The company will work with the appropriate health authorities on the implications on the program. The company expects to initiate its first study in pediatric patients in the fourth quarter of 2013 or the first quarter of 2014.

- **BMN 190 for LINCL (Batten disease):** BioMarin filed a CTA in the first quarter of 2013 and expects to begin enrolling the study in the third quarter of 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 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 Adjusted EBITDA Reconciliation

The results for the three and six months ended June 30, 2013 and June 30, 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 June 30,		Six Months Ended June 30,		Year Ending December 31,
	NOTES	2013	2012	2013	2012	2013 Guidance

GAAP Net Loss		**$ (21.5)**	**$ (32.0)**	**$ (61.3)**	**$ (56.0)**	**$(185.0) - $(160.0)**
Interest expense, net		--	1.4	1.0	2.8	0.7
Provision for (benefit from) income taxes		1.2	(0.4)	(3.5)	(0.4)	(14.6)
Depreciation expense		6.6	7.2	12.7	14.5	27.0
Amortization expense		3.6	2.7	6.2	12.0	11.5
EBITDA Loss		**(10.1)**	**(21.1)**	**(44.9)**	**(27.1)**	**(160.4) - (135.4)**
Stock-based compensation expense		13.9	12.6	25.5	23.8	70.0
Contingent consideration expense	(1)	(3.8)	1.2	1.0	(3.9)	15.0
Material non-recurring:						
Debt conversion expense	(2)	--	--	10.4	--	10.4
Non-GAAP Adjusted EBITDA Loss		**$ 0.0**	**$ (7.3)**	**$ (8.0)**	**$ (7.2)**	**$(65.0) - $(40.0)**

(1) Represents the expense associated with the change 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 second quarter 2013 financial results today, Thursday, July 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: July 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: 13894023

Replay Dial-in Number: 855.859.2056
Replay International Dial-in Number: 404.537.3406
Conference ID: 13894023

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 III 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 Vimizim and our other product candidates; and actions by regulatory authorities, particularly actions related to Vimizim. 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 Vimizim and each of the other 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
June 30, 2013 and December 31, 2012
(In thousands of U.S. dollars, except share and per share amounts)

	June 30, 2013	December 31, 2012(1)
ASSETS	(unaudited)	

Current assets:		
Cash and cash equivalents	$175,445	$180,527
Short-term investments	225,116	270,211
Accounts receivable, net (allowance for doubtful accounts: $376 and $348, respectively)	115,063	109,066
Inventory	142,296	128,695
Current deferred tax assets	29,474	29,454
Other current assets	33,526	25,509
Total current assets	720,920	743,462
Noncurrent assets:		
Investment in BioMarin/Genzyme LLC	1,001	1,080
Long-term investments	123,905	115,993
Property, plant and equipment, net	282,002	284,473
Intangible assets, net	168,434	162,980
Goodwill	54,975	51,543
Long-term deferred tax assets	235,400	225,501
Other assets	14,883	16,611
Total assets	$1,601,520	$1,601,643
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$142,241	$147,068
Convertible debt	--	23,365
Total current liabilities	142,241	170,433
Noncurrent liabilities:		
Long-term convertible debt	109,822	324,859
Long-term contingent acquisition consideration payable	23,261	30,618
Long-term deferred tax liabilities	37,182	33,296
Other long-term liabilities	30,756	26,674
Total liabilities	343,262	585,880
Stockholders' equity:		
Common stock, $0.001 par value: 250,000,000 shares authorized at June 30, 2013 and December 31, 2012; 140,050,009 and 125,809,162 shares issued and outstanding at June 30, 2013 and December 31, 2012, respectively.	140	126
Additional paid-in capital	1,863,961	1,561,890
Company common stock held by Nonqualified Deferred Compensation Plan	(7,493)	(6,603)
Accumulated other comprehensive income (loss)	2,441	(202)
Accumulated deficit	(600,791)	(539,448)
Total stockholders' equity	1,258,258	1,015,763
Total liabilities and stockholders' equity	$1,601,520	$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 and Six Months Ended June 30, 2013 and 2012
(In thousands of U.S. dollars, except per share amounts)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2013	**2012**	**2013**	**2012**
REVENUES:				
Net product revenues	$132,400	$122,991	$259,744	$239,230
Collaborative agreement revenues	889	423	1,024	519
Royalty and license revenues	3,521	605	3,970	919
Total revenues	136,810	124,019	264,738	240,668
OPERATING EXPENSES:				
Cost of sales (excludes amortization of certain acquired intangible assets)	22,567	23,574	43,067	40,679
Research and development	85,661	77,812	169,404	151,646
Selling, general and administrative	50,656	51,539	101,706	96,787
Intangible asset amortization and contingent consideration	(2,022)	2,048	3,534	4,376
Total operating expenses	156,862	154,973	317,711	293,488
LOSS FROM OPERATIONS	(20,052)	(30,954)	(52,973)	(52,820)
Equity in the income (loss) of BioMarin/Genzyme LLC	(163)	102	(564)	(632)
Interest income	650	536	1,368	1,041
Interest expense	(603)	(1,925)	(2,328)	(3,872)
Debt conversion expense	--	--	(10,420)	--
Other income (expense)	(123)	(176)	105	(140)
LOSS BEFORE INCOME TAXES	(20,291)	(32,417)	(64,812)	(56,423)
Provision for (benefit from) income taxes	1,242	(411)	(3,469)	(445)
NET LOSS	$(21,533)	$(32,006)	$(61,343)	$(55,978)
NET LOSS PER SHARE, BASIC	$(0.15)	$(0.27)	$(0.46)	$(0.48)
NET LOSS PER SHARE, DILUTED	$(0.16)	$(0.27)	$(0.46)	$(0.48)

Weighted average common shares outstanding, basic	139,400	117,912	133,716	116,496
Weighted average common shares outstanding, diluted	139,596	117,912	133,716	116,496
COMPREHENSIVE LOSS	$(20,247)	$(29,868)	$(58,700)	$(56,215)

STOCK-BASED COMPENSATION EXPENSE

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

	Three Months Ended June 30,		**Six Months Ended June 30,**	
	2013 (unaudited)	**2012 (unaudited)**	**2013 (unaudited)**	**2012 (unaudited)**
Cost of sales	$1,130	$1,335	$2,174	$2,207
Research and development	6,381	5,468	11,705	10,290
Selling, general and administrative	6,418	5,834	11,615	11,268
	$13,929	$12,637	$25,494	$23,765

CONTACT: Investors and Media:

Debra Charlesworth

BioMarin Pharmaceutical Inc.

(415) 455-7451
