BioMarin InvestorRoom

BioMarin Announces Third Quarter 2012 Financial Results

Steady Commercial Growth Supports Advancing Pipeline
GALNS Phase 3 Results Expected in 4Q 2012
Conference Call and Webcast to Be Held Today at 5:00 p.m. ET

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

	Q3 2012	Q3 2011	Percent Change
Total BioMarin Revenue	$ 128.1	$ 113.4	13.0%
Total Net Product Revenue	126.3	112.9	11.9%
Naglayzme Net Product Revenue	62.5	55.9	11.8%
Aldurazyme BioMarin Net Product Revenue	23.8	23.0	3.5%
Kuvan Net Product Revenue	36.4	30.5	19.3%
Firdapse Net Product Revenue	3.6	3.5	2.9%
GAAP Net Loss	(5.4)	(17.7)	
GAAP Net Loss per Share (basic and diluted)	$ (0.04)	$ (0.16)	
Non-GAAP Adjusted EBITDA Income	11.1	4.3	

SAN RAFAEL, Calif., Oct. 25, 2012 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced financial results for the third quarter of 2012. GAAP net loss was $5.4 million ($0.04 per share) for the third quarter of 2012, compared to GAAP net loss of $17.7 million ($0.16 per share) for the third quarter of 2011. Non-GAAP adjusted EBITDA was $11.1 million for the third quarter of 2012, compared to non-GAAP adjusted EBITDA of $4.3 million for the third quarter of 2011. The reduced GAAP net loss and the increased non-GAAP adjusted EBITDA for the third quarter of 2012 compared to the third quarter of 2011 was primarily due to increased net product revenue partially offset by a smaller increase in research and development expenses.

GAAP net loss for the nine months ended September 30, 2012 was $61.3 million ($0.52 per share), compared to GAAP net loss of $27.1 million ($0.24 per share) for the nine months ended September 30, 2011. Non-GAAP adjusted EBITDA was $3.9 million for the nine months ended September 30, 2012, compared to non-GAAP adjusted EBITDA of $37.4 million for the nine months ended September 30, 2011. The increased GAAP net loss and the reduced non-GAAP adjusted EBITDA for the first three quarters of 2012 compared to the first three quarters of 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 September 30, 2012, BioMarin had cash, cash equivalents and short and long-term investments totaling $533.2 million, as compared to $524.6 million on June 30, 2012.

"During the third quarter, the commercial portfolio showed continued steady growth, and we announced the advancement of two important pipeline programs. PEG-PAL will advance as our next pivotal Phase 3 program in the second quarter of next year and BMN-111 for achondroplasia will advance to a Phase 2 program in mid 2013," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "The next major inflection point for the company is the readout of the GALNS pivotal Phase 3 study. We also expect to release Phase 2 results for our BMN-701 Pompe program in the first quarter of 2013 and additional data on our BMN-673 PARP-inhibitor program before year end."

Net Product Revenue (in millions)

	Three Months Ended September 30,				Nine Months Ended September 30,			
	2012	2011	$ Change	% Change	2012	2011	$ Change	% Change
Naglazyme [1]	$ 62.5	$ 55.9	$ 6.6	11.8%	$ 193.9	$ 176.8	$ 17.1	9.7%
Kuvan [2]	36.4	30.5	5.9	19.3%	103.1	86.0	17.1	19.9%
Firdapse	3.6	3.5	0.1	2.9%	10.8	9.8	1.0	10.2%

(1) Changes in foreign currency rates, net of hedges, had a negative $0.2 million and a negative $1.4 million impact on Naglazyme sales in the three and nine months ended September 30, 2012, compared to the same periods in 2011, respectively. 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 16.5 percent in the third quarter of 2012 compared to the third quarter of 2011.

	Three Months Ended September 30,				Nine Months Ended September 30,			
	2012	2011	$ Change	% Change	2012	2011	$ Change	% Change
Aldurazyme revenue reported by Genzyme [3]	$ 48.3	$ 46.3	$ 2.0	4.3%	$ 140.0	$ 136.4	$ 3.6	2.6%
Royalties due from Genzyme	19.7	19.0	0.7		56.6	53.0	3.6	
Incremental product transfer revenues [4]	4.1	4.0	0.1		1.1	6.0	(4.9)	
Total Aldurazyme net product revenues	$ 23.8	$ 23.0	$ 0.8		$ 57.7	$ 59.0	$ (1.3)	

(3) The total number of Aldurazyme patients increased 9.5 percent in the third quarter of 2012 as compared to the third quarter of 2011.

(4) 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.

2012 Guidance

Revenue Guidance ($ in millions)

Item	2012 Guidance	Previous 2012 Guidance
Total BioMarin Revenues	$475 to $510	Unchanged
Total Net Product Revenues	$470 to $505	Unchanged
Naglazyme Net Product Revenue	$250 to $265	Unchanged
Kuvan Net Product Revenue	$130 to $140	Unchanged
Aldurazyme Net Product Revenue to BioMarin	$81 to $87	Unchanged
Firdapse Net Product Revenue	$13 to $17	Unchanged

Selected Income Statement Guidance ($ in millions)

Item	2012 Guidance	Previous 2012 Guidance
Cost of Sales (% of Total Revenue)	17% to 18%	Unchanged
Selling, General and Admin. Expense	$195 to $205	Unchanged
Research and Development Expense	$285 to $295	Unchanged
Amortization and Contingent Consideration	$19	Unchanged
Income Tax Expense	$(5)	$ --
GAAP Net (Loss)	$(100) to $(110)	$(105) to $(115)
Stock Compensation Expense	$50	Unchanged
Non-GAAP Adjusted EBITDA	$(5) to $5	Unchanged

Non GAAP Net Cash Flow (Usage)	$(20) to $(30)	$(40) to $(50)
Cash Balance*	$495 to $505	$475 to $485

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

Anticipated Upcoming Milestones

4Q 2012: Results for Phase 3 trial for GALNS for MPS IVA
4Q 2012: Update on Phase 1/2 trial for BMN-673 for solid tumors
1Q 2013: Market authorization application filing for GALNS for MPS IVA
1Q 2013: Results for Phase 1/2 trial for BMN-701 for Pompe disease
1H 2013: IND filing for BMN-190 for LINCL (Batten disease)
2Q 2013: Initiation of Phase 3 trial for PEG-PAL for PKU
2Q 2013: Results for PKU-016 Kuvan neurocognitive study
Mid 2013: Initiation of Phase 2 trial for BMN-111 for achondroplasia
2H 2013: Potential initiation of Phase 3 trial for BMN-673 for solid tumors
4Q 2013: Potential FDA approval of GALNS for MPS IVA

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

- **GALNS for MPS IVA:** The company is on track to report top-line results in the current quarter. Two of the three ancillary studies are fully enrolled: a clinical trial for patients under five years of age and a cardiopulmonary study to more completely document the health benefits of GALNS. A study in patients with limited ambulation is expected to complete enrollment this quarter.

Mid-Stage Clinical Programs

- **PEG-PAL for PKU:** The company expects to initiate a pivotal Phase 3 study in the second quarter of 2013, following an anticipated end of Phase 2 meeting with the FDA in the first quarter of 2013. The preliminary Phase 3 design includes an open-label study to evaluate safety and blood Phe levels in naïve patients and a randomized controlled study of the Phase 2 extension study patients to evaluate blood Phe levels and psychiatric and executive function endpoints.

- **Kuvan Outcomes Study:** PKU-016, a randomized, placebo-controlled, 13-week outcomes study in patients treated with Kuvan is fully 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 the second quarter of 2013.

Early-Stage Clinical Programs

- **BMN-701 for Pompe Disease:** The company has completed the enrollment of 16 patients in the 20 mg/kg cohort. Top-line results for the Phase 1/2 trial are expected in the first quarter of 2013.

- **BMN-673 (PARP inhibitor):** Over 20 patients have been dosed in the Phase 1/2 open-label trial on once daily, orally administered BMN-673 for advanced or recurrent solid tumors. An update on this program is expected in the fourth quarter of 2012. A Phase 3 trial in advanced or recurrent solid tumors could start enrolling by the end of 2013.

- **BMN-111 for Achondroplasia:** The company expects to initiate a Phase 2 trial in patients in mid 2013.

Preclinical Programs

- **BMN-190 for LINCL (Batten disease):** BioMarin expects to file the IND for BMN-190 in the first half 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 and nine months ended September 30, 2012 and September 30, 2011 and financial guidance for the year ending December 31, 2012 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 income 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 September 30,		Nine Months Ended September 30,		
	NOTES	2012	2011	2012	2011	Year Ending December 31, 2012 Guidance
GAAP Net Loss		**$ (5.4)**	**$ (17.7)**	**$ (61.3)**	**$ (27.1)**	**$(110.0) -- $(100.0)**
Interest expense, net		1.1	1.4	3.9	4.2	5.0
Income tax expense (benefit)		(6.4)	(2.1)	(6.8)	6.6	(5.0)
Depreciation		6.4	6.0	21.0	18.7	30.0
Amortization		2.7	1.0	14.5	2.9	11.0
EBITDA Income (Loss)		**(1.6)**	**(11.4)**	**(28.7)**	**5.3**	**(69.0) - (59.0)**
Stock-based compensation		12.1	11.6	35.9	32.6	50.0
Contingent consideration	(1)	0.6	2.2	(3.3)	(2.4)	14.0
Material non-recurring Convertible debt exchange	(2)	--	1.9		1.9	
Non-GAAP Adjusted EBITDA Income (Loss)		**$ 11.1**	**$ 4.3**	**$ 3.9**	**$ 37.4**	**$(5.0) -- $5.0**

(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.

(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 and uses the non-GAAP adjusted EBITDA methodology in establishing corporate goals for internal compensation programs.

Conference Call Details

BioMarin will host a conference call and webcast to discuss third quarter 2012 financial results today, Thursday, October 25, at 5:00 p.m. ET. This event can be accessed on the investor section of the BioMarin website at www.BMRN.com.

Date: October 25, 2012
Time: 5:00 p.m. ET
U.S. / Canada Dial-in Number: 877.303.6313
International Dial-in Number: 631.813.4734
Conference ID: 38161058

Replay Dial-in Number: 855.859.2056
Replay International Dial-in Number: 404.537.3406
Conference ID: 38161058

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
September 30, 2012 and December 31, 2011
(In thousands of U.S. dollars, except share and per share amounts)

	September 30, 2012	December 31, 2011(1)
ASSETS	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 181,330	$ 46,272
Short-term investments	245,125	148,820
Accounts receivable, net (allowance for doubtful accounts: $339 and $513, respectively)	117,290	104,839
Inventory	120,825	130,118
Other current assets	54,816	39,753
Total current assets	719,386	469,802
Investment in BioMarin/Genzyme LLC	848	559
Long-term investments	106,741	94,385
Property, plant and equipment, net	273,724	268,971
Intangible assets, net	165,624	180,277
Goodwill	51,543	51,543
Long-term deferred tax assets	229,771	222,649
Other assets	19,739	15,495
Total assets	$ 1,567,376	$ 1,303,681
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable and accrued liabilities	$ 111,489	$ 94,125
Convertible debt	23,440	--
Total current liabilities	134,929	94,125
Long-term convertible debt	324,861	348,329
Other long-term liabilities	92,392	88,179
Total liabilities	552,182	530,633
Stockholders' equity:		
Common stock, $0.001 par value: 250,000,000 shares authorized at September 30, 2012 and December 31, 2011: 123,790,150 and 114,789,732 shares issued and	124	115

outstanding at September 30, 2012 and December 31, 2011, respectively.		
Additional paid-in capital	1,505,776	1,197,082
Company common stock held by Nonqualified Deferred Compensation Plan	(6,603)	(3,935)
Accumulated other comprehensive income	2,333	4,887
Accumulated deficit	(486,436)	(425,101)
Total stockholders' equity	1,015,194	773,048
Total liabilities and stockholders' equity	$ 1,567,376	$ 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 and Nine Months Ended September 30, 2012 and 2011
(In thousands of U.S. dollars, except per share amounts)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2012	**2011**	**2012**	**2011**
REVENUES:				
Net product revenues	$ 126,310	$ 112,891	$ 365,540	$ 331,583
Collaborative agreement revenues	1,210	97	1,729	375
Royalty and license revenues	597	437	1,516	1,554
Total revenues	128,117	113,425	368,785	333,512
OPERATING EXPENSES:				
Cost of sales (excludes amortization of certain acquired intangible assets)	24,619	22,445	65,298	62,504
Research and development	66,209	58,577	217,855	156,466
Selling, general and administrative	46,337	44,880	143,124	126,969
Intangible asset amortization and contingent consideration	1,443	3,040	5,819	28
Total operating expenses	138,608	128,942	432,096	345,967
LOSS FROM OPERATIONS	(10,491)	(15,517)	(63,311)	(12,455)
Equity in the loss of BioMarin/Genzyme LLC	(336)	(608)	(968)	(1,817)
Interest income	778	722	1,819	2,302
Interest expense	(1,837)	(2,168)	(5,709)	(6,531)
Debt conversion expense	--	(1,896)	--	(1,896)
Other income and (expense)	125	(264)	(15)	(114)
LOSS BEFORE INCOME TAXES	(11,761)	(19,731)	(68,184)	(20,511)
Provision for (benefit from) income taxes	(6,404)	(2,078)	(6,849)	6,590
NET LOSS	$ (5,357)	$ (17,653)	$ (61,335)	$ (27,101)
NET LOSS PER SHARE, BASIC AND DILUTED	$ (0.04)	$ (0.16)	$ (0.52)	$ (0.24)
Weighted average common shares outstanding, basic and diluted	123,434	112,290	118,810	111,358

COMPREHENSIVE LOSS	$ (7,674)	$ (10,426)	$ (63,889)	$ (25,985)

STOCK-BASED COMPENSATION EXPENSE

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2012	2011	2012	2011
	(unaudited)	(unaudited)	(unaudited)	(unaudited)
Cost of sales	$ 1,327	$ 1,334	$ 3,535	$ 3,864
Research and development	5,060	4,372	15,351	12,070
Selling, general and administrative	5,752	5,912	17,021	16,673
	$ 12,139	$ 11,618	$ 35,907	$ 32,607

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