BioMarin Announces Third Quarter 2003 Financial Results

Conference Call and Webcast to be Held Today at 12:00 PM ET (18:00 CET)

PRNewswire-FirstCall
NOVATO, Calif.

BioMarin Pharmaceutical Inc. today announced financial results for its third quarter ended September 30, 2003. The net loss was $21.3 million ($0.33 per share) for the third quarter of 2003 compared to $17.1 million ($0.32 per share) for the third quarter of 2002. The net loss was $50.1 million ($0.81 per share) for the nine months ended September 30, 2003 compared to $58.1 million ($1.10 per share) for the nine months ended September 30, 2002.

As of September 30, 2003 BioMarin had cash and cash equivalents of approximately $242 million.

Fredric D. Price, Chairman and Chief Executive Officer of BioMarin said, "From an operational point of view, our programs are on track; from a strategic perspective, our research, clinical and business development projects address significant unmet patient needs; and financially, we have the resources necessary to drive our products towards commercialization."

The net loss in the third quarter of 2003 was due primarily to: market launch, clinical and regulatory activities associated with the company's first approved product, Aldurazyme(R) (laronidase), for the treatment of mucopolysaccharidosis I (MPS I); ongoing Phase 3 clinical development of Aryplase(TM) (arylsulfatase B) for mucopolysaccharidosis VI (MPS VI) and expenses related to the Phase 3a clinical trial of Neutralase(TM) and the

termination of this program.

Sales of Aldurazyme by BioMarin/Genzyme LLC were $3.4 million for the third quarter and $4.9 million for the nine months ended September 30, 2003 (Aldurazyme was launched on May 15, 2003). BioMarin's 50 percent share of the loss from BioMarin/Genzyme LLC was $4.5 million for the third quarter and $16.6 million for the nine months ended September 30, 2003.

Recent Events

-- On September 22, 2003, BioMarin announced that it halted the Phase 3a trial of Neutralase in coronary artery bypass graft surgery and discontinued the Neutralase drug development program.

-- On September 4, 2003, BioMarin announced data from preclinical studies demonstrating that intrathecal injection of recombinant alpha-L-iduronidase can reduce carbohydrate storage in brain tissue in the canine model of MPS I.

-- Aldurazyme was recently approved for MPS I in Norway and Iceland; applications are pending in Canada, Australia, New Zealand and Israel.

Program Updates

-- Aldurazyme sales for 2003 are expected to be in the range of $10 million to $13 million. As of October 15, 2003, approximately 100 MPS I patients were receiving commercial treatment with Aldurazyme. BioMarin and Genzyme continue to make progress in both MPS I patient identification and product reimbursement efforts. Genzyme recently reiterated the estimate of MPS I prevalence of approximately 3,000 patients in the United States (US), European Union (EU), and Japan. The epidemiological studies supporting this estimate for MPS I prevalence are referenced below:

-- Meikle PJ, Hopwood JJ, Clague AE, Carey WF: Prevalence of lysosomal storage diseases. JAMA (1999) 281:249-54.

-- Lowry RB, Applegarth DA, Toone JR, Macdonald E, Thunem NY: An update on the frequency of mucopolysaccharide syndromes in British Columbia. Human Genetics (1990) 85:389-390.

-- Nelson J: Incidence of the mucopolysaccharidoses in Northern Ireland. Human Genetics (1997) 101:355-358.

-- Poorthuis BJHM, Wevers RA, Kleijer WJ, et al.: The frequency of lysosomal storage diseases in The Netherlands. Human Genetics (1999) 105:151- 156.

-- In October 2003, the company completed enrollment in the pivotal Phase 3 trial of Aryplase, an investigational enzyme replacement therapy for MPS VI. BioMarin expects to announce data from this trial in the second quarter of 2004, and pending positive data, remains on track to file for marketing authorization in the US and EU in the fourth quarter of 2004. BioMarin continues MPS VI patient identification efforts well in advance of potential commercialization. To date, the company has enrolled approximately 170 patients in its Phase 1, Phase 2, Phase 3, and disease survey studies. BioMarin estimates that there are approximately 1,100 MPS VI patients in the developed world. The epidemiological studies cited above also support this estimate of MPS VI prevalence.

-- BioMarin expects to complete patient enrollment in its Phase 1b trial of Vibrilase(TM), a topical enzyme therapy under investigation for the debridement of serious burns, by the end of 2003.

-- The company will provide an update of its phenylketonuria (PKU) program in the fourth quarter of 2003. PKU is a genetic disorder affecting approximately 50,000 patients in the US and EU for which no drug treatment is currently available.

Clinical Presentations at American Society of Human Genetics

-- Investigators will present long-term data from ongoing Phase 1 and Phase 2 studies of Aryplase as well as data from studies of Aldurazyme and MPS I at the 53rd Annual Meeting of the American Society of Human Genetics being held November 4-8, 2003, in Los Angeles, CA. The following presentations will be made:

Aryplase

-- Abstract # 192: A Phase 1 Randomized, Double-Blind, Two Dose Group Study of Recombinant Human N-acetylgalactosamine-4-sulfatase (rhASB) Enzyme Replacement Therapy in Patients with Mucopolysaccharidosis VI (Maroteaux-Lamy Syndrome): 96 Week Progress Report

-- Abstract # 2672: A Phase 2 Open-Label Clinical Study of Efficacy and Safety of Recombinant Human N-acetylgalactosamine-4-sulfatase (rhASB) Enzyme Replacement Therapy in Patients with Mucopolysaccharidosis VI (Maroteaux-Lamy Syndrome)

Aldurazyme

-- Abstract # 2667: Aldurazyme (laronidase) Enzyme Replacement Therapy in MPS I: 48-Week Extension Data

-- Abstract # 2669: Aldurazyme (laronidase) Enzyme Replacement Therapy in

MPS I: Preliminary Safety Data in Children Less Than 5 Years of Age

-- Abstract # 2660: Effects of Aldurazyme (laronidase) on Joint Mobility in MPS I

-- Abstract # 2675: The Clinical Benefit of Aldurazyme (laronidase) for the Treatment of MPS I

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-- Abstract # 2659:  MPS I Management and Treatment Guidelines
-- Abstract # 1656:  The MPS I Registry
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-- Abstract # 2677: Pharmacokinetics of Aldurazyme (laronidase) for the Treatment of MPS I

-- Abstract # 1631: Physical Performance Measure for Individuals with MPS I

-- Abstract # 1661: The Impact of MPS I on the Functional Status of Affected Individuals

Upcoming Corporate Presentations

-- BioMarin will present an overview of its business and product development programs at the following healthcare and biotechnology investment conferences in the fourth quarter of 2003:

-- The CIBC World Markets 14th Annual Healthcare Conference, November 10, 2003, The Plaza Hotel, New York City, NY

-- Harris Nesbitt Gerard Healthcare Investor Conference, December 11, 2003, Waldorf Astoria, New York City, NY

Financial Guidance

BioMarin is projecting no change from its September 22, 2003 guidance of cash burn and net loss for 2003 and 2004: Cash burn for 2003 will be between $74 million and $78 million, and will be between $69 million and $73 million for 2004. Net loss will be between $76 million and $78 million for 2003 and between $68 million and $70 million for 2004.

BioMarin defines cash burn as the net increase (or decrease) in cash excluding the effect of capital markets financing activities and the purchase and sale of short-term investments (each as determined in accordance with generally accepted accounting principles). BioMarin estimates total net increase in cash for the year ending December 31, 2003 to be approximately $131 million to $135 million. This estimate includes the aggregate net proceeds from its public offering of common stock, sales of common stock to an institutional investor and a convertible debt offering of approximately $209 million, and assumes that the net effect of the purchase and sale of short- term investments will be zero. BioMarin does not anticipate any additional capital market financing activities in the remainder of 2003 or in 2004. Therefore, assuming that the net effect of the purchase and sale of short-term investments will be zero, the estimated cash burn for 2004 is equal to the estimated total net decrease in cash for 2004.

BioMarin will host a conference call and webcast to discuss third quarter financial results today at 12:00 PM ET (18:00 CET). This event can be accessed on the BioMarin website at: http://investor.biomarinpharm.com/ .

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Date:  November 4, 2003
Time:  12:00 PM ET (18:00 CET)
U.S. & Canada Toll-free Dial in #:  1-800-915-4836
International Dial in #:  973-317-5319
Replay Toll-free Dial in #:  1-800-428-6051
Replay International Dial in #:  973-709-2089
Replay Code #:  308927
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BioMarin Pharmaceutical Inc. develops and commercializes enzyme therapies for serious, life-threatening diseases and conditions.

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: results of preclinical studies of alpha-L-iduronidase; results and progress related to clinical trials of Aryplase and Vibrilase, including expected timing, progress, enrollment and conduct of current and future trials; expectations about commercial development of, and actions of regulatory authorities with respect to Aldurazyme, including expected sales of Aldurazyme; preclinical development of a product candidate for the treatment of PKU; and patient registry activities. 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: the final analysis of results of past preclinical and clinical trials; results and timing of current and future preclinical and clinical trials; enrollment rates of current and future clinical trials; the content and timing of decisions by regulatory authorities, including decisions related to Aldurazyme, Aryplase and Vibrilase; the actions of BioMarin's joint venture partner Genzyme in commercializing Aldurazyme and the success of those actions; our continued ability to manufacture Aldurazyme to meet commercial demand and to manufacture Aryplase for clinical trials; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption "Factors That May Affect Future Results" in BioMarin's 2002 Annual Report on Form 10-K and the factors contained in BioMarin's reports on Forms 10-Q and 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's press releases and other company information are available online at www.BMRN.com . Information on BioMarin's website is not incorporated by reference into this press release.

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BioMarin Pharmaceutical Inc. and Subsidiaries

Consolidated Balance Sheets
(In thousands, except share and per share data)

	December 31, 2002 (1)	September 30, 2003 (unaudited)
Assets		
Current assets:		
Cash and cash equivalents	$33,638	$187,978
Short-term investments	40,340	53,911
Investment in and advances to BioMarin/Genzyme LLC	4,955	10,310
Other current assets	2,139	2,007
Total current assets	81,072	254,206
Property and equipment, net	28,206	23,993
Other assets	1,338	6,247
Total assets	$110,616	$284,446
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and accrued liabilities	$3,930	$13,399
Other current liabilities	2,917	2,844
Total current liabilities	6,847	16,243

Convertible debt	--	125,000
Other long-term liabilities	5,226	1,240
Total liabilities	12,073	142,483
Stockholders' equity:		
Common stock, $0.001 par value: 75,000,000 and 150,000,000 shares authorized at December 31, 2002 and September 30, 2003, respectively, 53,782,426 and 63,870,972 shares issued and outstanding December 31, 2002 and September 30, 2003, respectively	54	64
Additional paid-in capital	319,038	412,355
Warrants	5,219	5,219
Deferred compensation	(47)	--
Notes receivable from stockholders	(468)	--
Accumulated other comprehensive income (loss)	327	(24)
Accumulated deficit	(225,580)	(275,651)
Total stockholders' equity	98,543	141,963
Total liabilities and stockholders' equity	$110,616	$284,446

(1) December 31, 2002 balances were obtained from audited financial statements.

BioMarin Pharmaceutical Inc. and Subsidiaries

Consolidated Statements of Operations
For the Three Months Ended September 30, 2002 and 2003
(In thousands, except per share data, unaudited)

	Three Months Ended September 30,	
	2002	2003
Operating expenses:		
Research and development	$7,738	$14,505
General and administrative	3,739	1,662
Equity in the loss of BioMarin/Genzyme LLC	5,919	4,503
Total operating expenses	17,396	20,670
Loss from operations	(17,396)	(20,670)
Interest income	629	781
Interest expense	(123)	(1,402)

Net loss from continuing operations	(16,890)	(21,291)
Loss from discontinued operations	(219)	--
Loss on disposal of discontinued operations	(8)	--
Net loss	$(17,117)	$(21,291)
Net loss per share, basic and diluted:		
Net loss from continuing operations	$(0.32)	$(0.33)
Income from discontinued operations	--	--
Loss on disposal of discontinued operations	--	--
Net loss	$(0.32)	$(0.33)
Weighted average common shares outstanding	53,446	63,815

BioMarin Pharmaceutical Inc. and Subsidiaries

Consolidated Statements of Operations
For the Nine Months Ended September 30, 2002 and 2003
(In thousands, except per share data, unaudited)

	Nine Months Ended September 30,	
	2002	2003
Milestone revenue	$--	$12,100
Operating expenses:		
Research and development	20,105	37,227
General and administrative	10,484	8,921
Equity in the loss of BioMarin/Genzyme LLC	17,893	16,642
In-process research and development	11,223	--
Total operating expenses	59,705	62,790
Loss from operations	(59,705)	(50,690)
Interest income	2,033	1,787
Interest expense	(375)	(1,745)
Net loss from continuing operations	(58,047)	(50,648)
Income from discontinued operations	75	--
Gain (loss) on disposal of discontinued operations	(159)	577

Net loss	$(58,131)	$(50,071)
Net loss per share, basic and diluted:		
Net loss from continuing operations	$(1.10)	$(0.82)
Income from discontinued operations	--	--
Gain (loss) on disposal of discontinued operations	--	0.01
Net loss	$(1.10)	$(0.81)
Weighted average common shares outstanding	53,011	61,450

SOURCE: BioMarin Pharmaceutical Inc.

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