BioMarin Announces First Quarter 2004 Financial Results

Conference Call and Webcast to be Held Today at 12:00 p.m. EDT (18:00 CEST)

PRNewswire-FirstCall
NOVATO, Calif.

BioMarin Pharmaceutical Inc. today announced financial results for the first quarter of the fiscal year 2004. The company incurred a net loss of $19.9 million ($0.31 per share) for the quarter ended March 31, 2004 compared to $19.7 million ($0.35 per share) for the quarter ended March 31, 2003.

As of March 31, 2004, BioMarin had cash, cash equivalents, and short-term investments of approximately $191.2 million.

The net loss in the first quarter of 2004 was due primarily to: the continued clinical development of Phase 3 product candidate Aryplase(TM) for the treatment of mucopolysaccharidosis VI (MPS VI); BioMarin's share of the loss from BioMarin/Genzyme LLC related to Aldurazyme(R); and expenses related to the advancement of the company's phenylketonuria (PKU) clinical development program.

Fredric D. Price, Chairman and Chief Executive Officer of BioMarin stated, "We are pleased with the progress we have made this year in our transformation to a fully integrated, pediatric-focused biopharmaceutical company with commercial operations in the U.S. We anticipate completion of the recently announced Ascent Pediatrics transaction soon, and in this current quarter we will announce the results of the just completed Phase 3 trial of Aryplase for MPS VI. If the results from that trial are positive, we anticipate filing applications for approval of Aryplase in the U.S. and the E.U. in the fourth quarter of 2004."

First Quarter Highlights
Ascent Pediatrics Transaction

-- On April 20, 2004, BioMarin announced the signing of a definitive agreement with Medicis Pharmaceutical Corporation to obtain its pediatric business, Ascent Pediatrics, Inc. The transaction, which is expected to close in the second quarter of 2004, brings with it Orapred(R), a U.S. Food and Drug Administration (FDA) approved, patent-protected drug to treat asthma in children, two additional proprietary formulations of Orapred, and a U.S. pediatric-focused sales organization.
-- Excluding the non-cash charges associated with the transaction, BioMarin expects that the net loss for 2004 will be less than the previously announced guidance of a loss between $68 million to $70 million. The company expects that the net sales of Orapred will be approximately $42 million for the 12 months ending December 31, 2004. BioMarin will record all Orapred revenues beginning with the transaction closing.
-- BioMarin expects the loss for 2005 will decrease significantly due to the anticipated profits from Orapred and Aldurazyme, an enzyme replacement therapy for the treatment of mucopolysaccharidosis I (MPS I), marketed on a worldwide basis by Genzyme Corporation. BioMarin will provide more specific revised guidance for 2004 and 2005 during the third quarter of this year and at the end of this year, respectively.

Aldurazyme for MPS I
-- Sales of Aldurazyme by BioMarin/Genzyme LLC were $7.4 million for the first quarter of 2004. Previously announced sales guidance for the 12 months ending December 31, 2004, provided by Genzyme Corporation, remains unchanged at between $40 million and $44 million. Aldurazyme received marketing approval from the FDA and the European Commission in the second quarter of 2003 and since then, Aldurazyme has been approved in Norway, Iceland, Israel, and the Czech Republic, with additional applications pending in Canada, Australia, New Zealand, Russia, Korea, and Bulgaria.

Aryplase for MPS VI
-- BioMarin recently completed the Phase 3 trial of Aryplase for MPS VI

and expects to announce the results in the second quarter of 2004, following analysis of the data from the six clinical sites. If the data are positive, the company plans to file simultaneous applications for approval in the U.S. and European Union in the fourth quarter of 2004.

Phenoptin(TM) for PKU
-- On February 23, 2004, BioMarin announced the initiation of its PKU clinical program beginning with a pilot trial of tetrahydrobiopterin (6R-BH4). The company expects to announce data from this trial in the third quarter and, pending positive trial results, to initiate an additional clinical trial in late 2004.

Upcoming Presentations at Medical Conferences

Investigators will present data from ongoing studies of Aldurazyme for MPS I and Aryplase for MPS VI at the following three conferences:

3rd International Conference: Prospects in the Treatment of Rare Diseases, May 19-22, 2004, Trieste, Italy

Aryplase/ MPS VI
-- Enzyme Replacement Therapy for Skeletal Disease in Mucopolysaccharidosis Type VI

Immune Tolerance
-- Prospects for Treating Null-Genotyped Patients with Neurologic Disease Using Immune Tolerance and Intrathecal Enzyme Therapy

8th International Symposium on MPS and Related Diseases, June 10 - 13, 2004, Mainz, Germany

Aldurazyme/ MPS I
-- Aldurazyme(R) (laronidase) Enzyme Replacement Therapy in MPS I: Preliminary Data in Children Less Than Five Years of Age
-- The Attenuated ('Non-Hurler') Phenotype of Mucopolysaccharidosis: A Review of the Clinical Features in 29 Patients
-- MPS I Characteristics: Description of First Patients Entered into the MPS I Registry
-- Update on Clinical Trial Results in MPS I
-- The MPS I Registry: Centralised Data Collection to Outline the Natural History and Treatment of MPS I Disease
-- Successful Treatment of the Brain and Meninges with Immune Tolerance in Canine MPS I

Aryplase/ MPS VI
-- Update on Phase 1, 2, and 3 Clinical Trial Results of Aryplase in MPS VI
-- A Threshold Effect of Urinary Glycosaminoglycans and the Walk Test as Indicators of Disease Progression in a Survey of Subjects with Mucopolysaccharidosis VI (Maroteaux-Lamy Syndrome)
-- Long-Term Combined Therapy with Recombinant Human N-acetylgalactosamine-4-Sulfatase for Degenerative Joint Disease in Mucopolysaccharidosis VI Cats
-- Flexible Endoscopy in a 13-Year-Old Boy with MPS VI and Tracheostomy Because of Upper Airway Obstruction: Changes After 91 and 146 Weeks of a Phase I/II Enzyme Replacement Therapy with Recombinant Human Arylsulfatase B

36th European Human Genetics Conference, June 12 - 15, 2004, Munich, Germany

Aryplase/ MPS VI
-- Update on Phase 1, 2, and 3 Clinical Trial Results of Aryplase in MPS VI
-- A Threshold Effect of Urinary Glycosaminoglycans and the Walk Test as Indicators of Disease Progression in a Survey of Subjects with Mucopolysaccharidosis VI (Maroteaux-Lamy Syndrome)

Upcoming Corporate Presentations

BioMarin will present an overview of its business and product development programs at the following upcoming

healthcare and biotechnology investment conferences:

-- Rodman & Renshaw Global Healthcare Conference, May 12-14, 2004, London, United Kingdom
-- BioEquity Europe 2004, May 17-18, 2004, Edinburgh, United Kingdom
-- UBS 2004 Global Specialty Pharmaceutical Conference, May 24-25, 2004, New York City, NY
-- Pacific Growth Equities Life Sciences Growth Conference, June 9-10, 2004, San Francisco, CA
-- Needham & Co. Third Annual Biotechnology Conference, June 16-17, 2004, New York City, NY

BioMarin will host a conference call and webcast to discuss first quarter financial results today at 12:00 p.m. EDT (18:00 CEST). This event can be accessed on the BioMarin website at: http://investor.biomarinpharm.com/ .

Date: May 4, 2004
Time: 12:00 p.m. EDT (18:00 CEST)
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 #: 346222

BioMarin Pharmaceutical Inc. develops innovative biopharmaceuticals and commercializes therapeutics for serious pediatric diseases.

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the possible acquisition of the Ascent Pediatrics, Inc. business and the financial performance of that business if the transaction is completed; the financial performance of Aldurazyme and the company as a whole; the continued development and commercialization of Aldurazyme, Aryplase and Phenoptin; 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: actual completion of the proposed transaction; the successful integration of the sales force and the Orapred business into BioMarin's organization; results and timing of current and planned preclinical studies and clinical trials; the content and timing of decisions by the FDA, the European Commission and other regulatory authorities concerning each of the described products; the market for each of these products and particularly Aldurazyme and Orapred; actual sales of Aldurazyme and Orapred; the possible development of competing products; 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 2003 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.

Orapred(R) is a registered trademark of Ascent Pediatrics, Inc., a wholly owned subsidiary of Medicis Pharmaceutical Corporation.

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BioMarin Pharmaceutical Inc. and Subsidiaries
Consolidated Balance Sheets
(In thousands, except share and per share data)

	December 31, 2003 (1)	March 31, 2004 (unaudited)
Assets		

Current assets:		
Cash and cash equivalents	$121,406	$96,199
Short-term investments	84,951	94,999
Investment in and advances to BioMarin/Genzyme LLC	16,058	15,149
Other current assets	2,854	2,478
Total current assets	225,269	208,825
Property and equipment, net	25,154	24,447
Other assets	5,917	6,024
Total assets	$256,340	$239,296
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and accrued liabilities	$10,098	$12,850
Other current liabilities	2,717	2,311
Total current liabilities	12,815	15,161
Convertible debt	125,000	125,000
Other long-term liabilities	672	363
Total liabilities	138,487	140,524
Stockholders' equity:		
Common stock, $0.001 par value: 150,000,000 shares authorized; 64,156,285 and 64,273,601 shares issued and outstanding December 31, 2003 and March 31, 2004, respectively	64	64
Additional paid-in capital	414,110	414,929
Warrants	5,219	5,219
Deferred compensation	(145)	(101)
Accumulated other comprehensive loss	(17)	(16)
Accumulated deficit	(301,378)	(321,323)
Total stockholders' equity	117,853	98,772
Total liabilities and stockholders' equity	$256,340	$239,296

(1) December 31, 2003 balances were derived from the audited consolidated financial statements.

BioMarin Pharmaceutical Inc. and Subsidiaries
Consolidated Statements of Operations
For the Three Months Ended March 31, 2003 and 2004
(In thousands, except per share data, unaudited)

	Three Months Ended March 31,	
	2003	2004
Operating expenses:		
Research and development	$10,991	$13,887
General and administrative	2,799	3,689
Equity in the loss of BioMarin/Genzyme LLC	6,753	1,759
Total operating expenses	20,543	19,335
Loss from operations	(20,543)	(19,335)
Interest income	413	761
Interest expense	(130)	(1,371)
Net loss from continuing operations	(20,260)	(19,945)
Gain on disposal of discontinued operations	577	--
Net loss	$(19,683)	$(19,945)

Net loss per share, basic and diluted:		
Net loss from continuing operations	$(0.36)	$(0.31)
Gain on disposal of discontinued operations	0.01	--
Net loss	$(0.35)	$(0.31)
Weighted average common shares outstanding	56,964	64,225

SOURCE: BioMarin Pharmaceutical Inc.

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