

# BioMarin Announces Second Quarter 2004 Financial Results

**Second Quarter GAAP Net Loss of \$0.86 Per Share and Pro Forma Adjusted Net Loss of \$0.28 Per Share**

**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 second quarter of the fiscal year 2004. The company incurred a GAAP net loss of \$55.6 million (\$0.86 per share) for the second quarter ended June 30, 2004 compared to \$9.1 million (\$0.14 per share) for the second quarter ended June 30, 2003. The GAAP net loss was \$75.5 million (\$1.18 per share) for the six months ended June 30, 2004 compared to \$28.8 million (\$0.48 per share) for the six months ended June 30, 2003.

Excluding non-cash expenses related to the acquisition of the Ascent Pediatrics business, BioMarin's Pro Forma Adjusted net loss was \$18.0 million (\$0.28 per share) for the quarter and \$37.9 million (\$0.59 per share) for the six months ended June 30, 2004. The following table reconciles BioMarin's GAAP net loss to BioMarin's Pro Forma Adjusted net loss for the quarter and six months ended June 30, 2004:

	Three Months Ended June 30, 2004	Six Months Ended June 30, 2004
Net loss	\$(55,598)	\$(75,543)
Non-GAAP adjustments:		
Acquired in-process research and development	35,444	35,444
Amortization of acquired intangible assets	819	819
Imputed interest expense	1,108	1,108
Fair value inventory adjustment	252	252
Pro Forma Adjusted net loss	\$(17,975)	\$(37,920)
Pro Forma Adjusted net loss per share, basic and diluted	\$(0.28)	\$(0.59)
Weighted average common shares outstanding, basic and diluted	64,339	64,282

BioMarin's net loss in the second quarter of 2004 was due primarily to: non-cash expenses of \$37.6 million related to the acquisition of the Ascent Pediatrics business from Medicis Pharmaceutical Corporation; \$8.2 million in ongoing research and development expenses related to Aryplase(TM) for the treatment of mucopolysaccharidosis VI (MPS VI); BioMarin's 50 percent share of the loss from BioMarin/Genzyme LLC of \$1.7 million related to Aldurazyme(R); and research and development expenses of \$1.6 million related to the advancement of the company's phenylketonuria (PKU) clinical development program.

BioMarin recorded \$4.6 million in net sales of Orapred(R) for the second quarter ended June 30, 2004. BioMarin began recording sales of Orapred after the close of the acquisition of the Ascent Pediatrics business from Medicis Pharmaceutical Corporation, which occurred on May 18, 2004. According to IMS Health (source IMS: MPA), prescriptions of Orapred increased approximately 10 percent for the first half of 2004 over prescriptions for the same period in 2003.

Net sales of Aldurazyme recorded by BioMarin/Genzyme LLC were \$9.2 million for the second quarter ended June 30, 2004 compared to \$1.1 million for the second quarter ended June 30, 2003 (the product was launched by Genzyme in the United States in May 2003 and in European Union in June 2003). Net sales for the six months ended June 30, 2004 were \$16.6 million compared to \$1.5 million for the same period in 2003.

Louis Drapeau, Chief Financial Officer of BioMarin stated, "The integration of the Ascent Pediatrics business is proceeding as planned and we are on track to complete the transition from Medicis to BioMarin within three months of the closing of the transaction." Mr. Drapeau continued, "In the second half of the year, we look forward to filing for regulatory approval of Aryplase(TM) for MPS VI in the United States and the European Union

based on the positive Phase 3 results we recently announced, and to initiating an additional clinical trial with Phenoptin(TM) in PKU patients."

#### Second Quarter Program Highlights

##### Positive Clinical Data on 6R-BH4 for PKU

On July 20, 2004, BioMarin announced positive results from a clinical study of 6R-BH4 in PKU patients. 6R-BH4 is the active agent in Phenoptin, an investigational small molecule therapeutic for the treatment of PKU. The results demonstrated that 6R-BH4 reduced blood phenylalanine levels by at least 30 percent in 45 percent of the patients in the study, with a dose of 10 mg/kg for seven days. When the dose was increased to 20 mg/kg, Phe levels dropped by at least 30 percent in 61 percent of the patients. The average reduction in blood Phe in these patients was 50 percent and 64 percent, respectively. No serious side effects of 6R-BH4 were observed. The company also announced that it has received orphan drug designation for Phenoptin in both the United States and European Union. BioMarin expects to initiate an additional clinical study with Phenoptin in PKU patients by the end of 2004.

##### Positive Phase 3 Clinical Data on Aryplase for MPS VI

On June 3, 2004, BioMarin announced positive results from its Phase 3 clinical trial of Aryplase, an investigational enzyme replacement therapy for the treatment of MPS VI. The clinical trial demonstrated a statistically significant improvement in the primary endpoint of endurance ( $p=0.025$ ) and one of the secondary endpoints, urinary glycosaminoglycan reduction ( $p<0.001$ ), in patients receiving Aryplase compared to patients receiving placebo. In the three-minute stair climb endurance test, also a secondary endpoint, patients receiving Aryplase demonstrated an improvement ( $p=0.053$ ) over patients receiving placebo. BioMarin expects to file for marketing authorization for Aryplase in both the United States and European Union in the fourth quarter of 2004. BioMarin has identified approximately 250 patients with MPS VI, an increase of approximately 25 percent over the previously reported number of approximately 200 patients.

##### Successful Integration of the Ascent Pediatrics Business

On May 18, 2004, BioMarin completed the acquisition of the Ascent Pediatrics business from Medicis Pharmaceutical Corporation. The transaction provides BioMarin a sales force of approximately 70 people that call on pediatricians located throughout the United States. The sales force details Orapred, the leading product in its class for the treatment of acute exacerbations of asthma in children. The integration of the Ascent Pediatrics business is proceeding rapidly without interruption to the sales and marketing activities, as evidenced by the continued growth in Orapred prescriptions. The Orapred sales and marketing team will soon augment existing precommercialization efforts associated with the company's additional product candidates, beginning with Aryplase.

##### Increasing Sales of Aldurazyme for MPS I

Sales of Aldurazyme by BioMarin/Genzyme LLC were \$9.2 million for the second quarter of 2004. Aldurazyme sales have increased steadily since its launch in the second quarter of 2003. Sales were \$1.1 million, \$3.4 million, \$6.7 million, and \$7.4 million and \$9.2 million for the five consecutive quarters since launch. Previously announced sales guidance for the 12 months ending December 31, 2004 provided by Genzyme Corporation, BioMarin's joint venture partner responsible for sales and marketing of Aldurazyme, remains unchanged at between \$40 million and \$44 million. BioMarin and Genzyme recently received marketing authorization for Aldurazyme in Canada and Australia.

##### Updated Financial Guidance

BioMarin expects that net sales of Orapred will be approximately \$42 million for the full year of 2004, of which BioMarin will realize the net sales recorded since the close of the deal with Medicis on May 18, 2004. The company expects that its Pro Forma Adjusted net loss for 2004, excluding the \$37.6 million in non-cash expenses related to the Ascent Pediatrics transaction, will be approximately \$66 million to \$68 million, which is less than the previously projected \$68 million to \$70 million. On a GAAP basis, BioMarin expects that the net loss for 2004 will be approximately \$112 million to \$114 million. BioMarin expects to end 2004 with approximately \$85 million to \$90 million in cash, cash equivalents, and restricted cash.

##### Upcoming Presentations at Medical Conferences

Investigators will present data from clinical studies of Vibrilase for serious burns, Aldurazyme for MPS I, Aryplase for MPS VI, and Phenoptin for PKU at several medical conferences in the coming months. The following is a list of the abstracts submitted for presentation:

The 12th Congress of the International Society for Burn Injuries, August 22 - August 26, 2004, Yokohama, Japan

- Clinical Development of Vibrilase(TM) (vibriolysin) for Burn Wound Debridement: Evaluation of Safety in a Phase 1a Study of Irritation and Contact Sensitization, and a Phase 1b Study of Partial-Thickness Burn Wound Debridement

The Society for Inborn Errors of Metabolism (SSIEM) 41st Annual Symposium, August 31 - September 3, 2004, Amsterdam, the Netherlands

- A Five Year Study of Aldurazyme for Treatment of MPS I
- Enzyme Replacement in Two Patients with MPS I
- Monthly Intrathecal Enzyme Therapy for Canine MPS I
- Update on Clinical Trial Results in MPS VI
- Multiple Dose Study of Tetrahydrobiopterin (BH4) in Phenylketonuria

The American Academy of Pediatrics 2004 National Conference and Exhibition, October 9 - 13, 2004, San Francisco, CA

- The Important Role of Pediatricians in Recognizing Symptom Clusters in Patients with Mucopolysaccharidosis I

American Society of Human Genetics Annual Meeting 2004, October 26 - 30, 2004, Toronto, Canada

- A Five Year Study of Aldurazyme for Treatment of MPS I
- The MPS I Registry: Centralized Data Collection to Delineate the Natural History and Treatment of MPS I
- Monthly Intrathecal Enzyme Replacement Therapy Improves Lysosomal Storage in the Brain and Spinal Cord in Canine MPS I
- A Phase 3, Randomized, Double-Blind, Placebo-Controlled, Multicenter, Multinational Clinical Study of Recombinant Human N-acetylgalactosamine 4-Sulfatase (rhASB) in Patients with Mucopolysaccharidosis VI

Upcoming Corporate Presentations

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

- UBS Global Life Sciences Conference, September 27 - 30, 2004, New York City, New York
- Rodman & Renshaw 6th Annual Healthcare Conference, October 26 - 28, 2004, New York City, New York
- CIBC World Markets 15th Annual Healthcare Conference, November 8 -10, 2004, New York City, New York

BioMarin will host a conference call and webcast to discuss second 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.biopharm.com/> .

Date: August 10, 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 #: 363459

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 performance of BioMarin's product Orapred and BioMarin/Genzyme LLC's product Aldurazyme; the financial performance of 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: the successful integration of the sales force, sales operations and the Orapred business into BioMarin's organization; our joint venture partner's success in continuing the commercialization of Aldurazyme; results and timing of current and planned preclinical studies and clinical trials; 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; 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](http://www.BMRN.com). Information on BioMarin's website is not incorporated by reference into this press release.

NOTE: Aldurazyme(R) is a registered trademark of BioMarin/Genzyme LLC. All rights reserved.

Orapred(R) is a registered trademark of Medicis Pediatrics, Inc., and is used under license.

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

	December 31, 2003 (1)	June 30, 2004 (unaudited)
<b>Assets</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$121,406	\$48,247
Short-term investments	84,951	80,549
Restricted cash	--	25,124
Accounts receivable	--	4,612
Inventory	--	2,074
Investment in and advances to BioMarin/Genzyme LLC	16,058	18,675
Other current assets	2,854	2,306
<b>Total current assets</b>	<b>225,269</b>	<b>181,587</b>
Property and equipment, net	25,154	27,000
Acquired intangible assets and goodwill	--	133,090
Other assets	5,917	5,739
<b>Total assets</b>	<b>\$256,340</b>	<b>\$347,416</b>
<b>Liabilities and Stockholders' Equity</b>		
<b>Current liabilities:</b>		
Accounts payable and accrued liabilities	\$10,098	\$20,650
Current portion of acquisition obligation, net of discount	--	58,228
Other current liabilities	2,717	2,392
<b>Total current liabilities</b>	<b>12,815</b>	<b>81,270</b>
Convertible debt	125,000	125,000
Equipment and facility loan	--	3,778
Long-term portion of acquisition obligation, net of discount	--	93,474
Other long-term liabilities	672	704
<b>Total liabilities</b>	<b>138,487</b>	<b>304,226</b>
<b>Stockholders' equity:</b>		
Common stock, \$0.001 par value: 150,000,000		

shares authorized; 64,156,285 and 64,366,988 shares issued and outstanding December 31, 2003 and June 30, 2004, respectively	64	64
Additional paid-in capital	414,110	420,577
Warrants	5,219	85
Deferred compensation	(145)	(58)
Accumulated other comprehensive loss	(17)	(557)
Accumulated deficit	(301,378)	(376,921)
 Total stockholders' equity	 117,853	 43,190
 Total liabilities and stockholders' equity	 \$256,340	 \$347,416

(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 and Six Months Ended June 30, 2003 and 2004  
(In thousands, except per share data, unaudited)

	Three Months Ended		Six Months Ended	
	June 30,		June 30,	
	2003	2004	2003	2004
Net product sales	\$--	\$4,563	\$--	\$4,563
Milestone revenue	12,100	--	12,100	--
Total revenue	12,100	4,563	12,100	4,563
Operating expenses:				
Cost of sales	--	613	--	613
Research and development	11,731	12,292	22,722	26,179
Selling, general and administrative	4,460	7,539	7,259	11,228
Amortization of acquired intangible assets	--	819	--	819
Acquired in-process research and development	--	35,444	--	35,444
Equity in the loss of BioMarin/ Genzyme LLC	5,386	1,699	12,139	3,458
Total operating expenses	21,577	58,406	42,120	77,741
Loss from operations	(9,477)	(53,843)	(30,020)	(73,178)
Interest income	593	663	1,006	1,424
Interest expense	(213)	(2,418)	(343)	(3,789)
Net loss from continuing operations	(9,097)	(55,598)	(29,357)	(75,543)
Gain on disposal of discontinued operations	--	--	577	--
Net loss	\$(9,097)	\$(55,598)	\$(28,780)	\$(75,543)
Net loss per share, basic and diluted:				
Net loss from continuing operations	\$(0.14)	\$(0.86)	\$(0.49)	\$(1.18)

Gain on disposal of discontinued operations	--	--	0.01	--
Net loss	\$(0.14)	\$(0.86)	\$(0.48)	\$(1.18)
Weighted average common shares outstanding, basic and diluted	63,494	64,339	60,247	64,282
Pro forma adjusted net loss (1)	\$(9,097)	\$(17,975)	\$(28,780)	\$(37,920)
Pro forma adjusted net loss per share	\$(0.14)	\$(0.28)	\$(0.48)	\$(0.59)

(1) Pro Forma Adjusted net loss excludes non-cash expenses related to the acquisition of the Ascent Pediatrics business totaling \$37.6 million. The non-cash expenses include in-process research and development, amortization of acquired intangible assets, imputed interest expense and a fair value inventory adjustment. Please refer to the body of the press release dated August 10, 2004 for discussion of Pro Forma Adjusted net loss as a non-GAAP financial measure.

SOURCE: BioMarin Pharmaceutical Inc.

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Web site: <http://www.bmrn.com/>

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