

# BioMarin Halts Phase 3a Study of Neutralase in CABG and Terminates the Neutralase Drug Development Program

**BioMarin Spending Projected to Decrease in Both 2003 and 2004  
Conference Call at 9:00 AM ET (15:00 CET)**

PRNewswire-FirstCall  
NOVATO, Calif.

BioMarin Pharmaceutical Inc. announced that it halted its Phase 3a study of Neutralase(TM) for the reversal of anticoagulation by heparin in primary Coronary Artery Bypass Graft (CABG) surgery and that it has terminated its Neutralase program for all indications.

The decision to halt the Phase 3 study resulted from a recommendation from an independent Data Safety Monitoring Board (DSMB) and was based on a review of data from enrolled patients, which indicated with high probability that Neutralase would not demonstrate favorable safety and efficacy. Given the expected risk/benefit profile for Neutralase, BioMarin has decided to stop development of the drug for all indications.

As a result of the planned reductions in spending related to the discontinuation of Neutralase development, BioMarin has lowered its projected net loss by \$5 million for 2003 and by \$13 million for 2004. Revised estimates for both net loss and cash burn for 2003 and 2004 are included below, as are previous forecasts for purposes of comparability:

Changes to 2003 and 2004 Forecast (\$ in millions)

Forecast	2003		2004	
	Current	Previous	Current	Previous
Net Loss	\$76-\$78	\$81-\$83	\$68-\$70	\$81-\$83
Cash Burn	\$74-\$78	\$80-\$84	\$69-\$73	\$80-\$84

As of June 30, 2003, BioMarin had approximately \$265 million in cash and cash equivalents.

Fredric D. Price, Chairman and Chief Executive Officer of BioMarin stated, "While we are disappointed that Neutralase will not become a successful product, it is better to have learned this relatively early in its development at BioMarin. We have planned BioMarin's product portfolio carefully, and have adequate resources for our other promising product opportunities, including Aryplase(TM), which is in a pivotal Phase 3 trial."

BioMarin's portfolio includes product candidates for genetic diseases, including Aryplase for mucopolysaccharidosis VI that is in a Phase 3 clinical trial expected to be completed at the end of the first quarter of 2004, and a product candidate for Phenylketonuria (PKU) that the company expects to advance into clinical-stage development in 2004. Vibrilase(TM), an enzyme product candidate for treatment of serious burns, is nearing completion of a Phase 1b study, and NeuroTrans(TM) is in preclinical testing as a method to deliver both enzymes and other therapeutic molecules to the brain via traditional intravenous infusion. Aldurazyme(R) for mucopolysaccharidosis I is approved in both the United States and European Union. Aldurazyme is manufactured by BioMarin and is marketed by BioMarin's joint venture partner, Genzyme Corporation.

BioMarin will host a conference call and webcast to discuss the decision to terminate Neutralase development today at 9:00 AM ET (15:00 CET). This event can be accessed on the BioMarin website at:

<http://investor.biopharm.com/>.

Date: September 22, 2003  
Time: 9:00 AM ET (15:00 CET)  
U.S. & Canada Toll-free Dial-in #: 1-888-391-0090  
International Dial-in #: 415-537-1988  
Replay Toll-free Dial-in #: 1-800-633-8625  
Replay International Dial-in #: 402-977-9141  
Replay Code #: 21161394

BioMarin develops and commercializes therapeutic enzyme products to treat serious, life-threatening diseases and conditions.

BioMarin defines cash burn as net cash flow for a fiscal year (determined in accordance with generally accepted accounting principles) less all proceeds from capital markets financing activities. BioMarin's proceeds from all capital markets financing activities in its fiscal year 2003 through the date of this release are \$209.4 million.

#### Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the net loss and cash burn of BioMarin for fiscal years 2003 and 2004, the cessation of clinical trials of Neutralase and the cessation of the development program for Neutralase for all indications, resources for product candidates, results of clinical trials of Aryplase as well as trials for a product for PKU and for Vibrilase, and expected timing and progress of current and future trials. 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 actual results of operations of BioMarin for its fiscal years 2003 and 2004, including without limitation actual revenues and expenses and cash flows; the final analysis of results of past clinical trials; results and timing of current and future clinical trials; the content and timing of decisions by regulatory authorities; 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 <http://www.bmrn.com/>. Information on BioMarin's website is not incorporated by reference into this press release.

#### Contacts:

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

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