BioMarin Announces Marketing Approval for Aldurazyme in Japan

PRNewswire-FirstCall
NOVATO, Calif.

BioMarin Pharmaceutical Inc. (Nasdaq and SWX: BMRN) announced today that Japan's Ministry of Health, Labor, and Welfare (MHLW) has granted marketing authorization for Aldurazyme(R) (laronidase), the first specific treatment approved in Japan for patients with the genetic disease mucopolysaccharidosis I (MPS I). Aldurazyme was approved in Japan as an orphan drug.

"I am very pleased that enzyme replacement therapy with Aldurazyme has finally been approved in Japan for the treatment of MPS I," stated Yoshikatsu Eto, M.D., Chairman and Professor in the Department of Pediatrics at Tokyo Jikei University Hospital located in Tokyo, Japan. "This is an important milestone for Japanese MPS I patients and their treating physicians who now have an effective treatment for this debilitating and life-threatening disease."

Aldurazyme was developed in partnership between BioMarin and Genzyme and was approved in the United States and the European Union in April and May 2003, respectively. Pursuant to the joint venture, BioMarin is responsible for manufacturing Aldurazyme and Genzyme is responsible for its commercialization. All expenses and profits and are shared equally between the companies.

About MPS I

MPS I is a progressive, debilitating and fatal genetic disease caused by a deficiency of the enzyme alpha-L-iduronidase. This deficiency leads to the accumulation of complex carbohydrates in the lysosomes of cells, leading to the progressive dysfunction of cellular, tissue and organ systems. Resulting symptoms, which span a spectrum of severity, can include impaired cardiac and pulmonary function, delayed physical development, skeletal and joint deformities, reduced endurance, and in some cases, delayed mental function. A majority of patients die before adulthood from complications of the disease.

About Aldurazyme

Aldurazyme(R) (laronidase) is indicated in the United States for patients with Hurler and

Hurler-Scheie forms of MPS I and for patients with the Scheie form who have moderate to severe symptoms. The risks and benefits of treating mildly affected patients with the Scheie form have not been established. Aldurazyme has been shown to improve pulmonary function and walking capacity. Aldurazyme has not been evaluated for effects on central nervous system manifestations of the disorder.

The most common adverse reactions associated with Aldurazyme treatment in clinical studies were upper respiratory tract infection, rash, and injection site reaction. The most common adverse reactions requiring intervention were infusion-related reactions, including flushing, fever, headache, and rash. The most serious adverse reaction reported with Aldurazyme was an anaphylactic reaction, which occurred in one patient approximately three hours after the start of the infusion. The reaction consisted of urticaria and airway obstruction. Resuscitation required an emergency tracheostomy. This patient's pre-existing MPS I-related upper airway obstruction may have contributed to the severity of this reaction. Approximately 91 percent of patients treated with Aldurazyme were positive for antibodies to laronidase. The clinical significance of antibodies to Aldurazyme is not known. There are no known contraindications to the use of Aldurazyme. Aldurazyme is available by prescription only. For more information on Aldurazyme, please see full prescribing information at www.aldurazyme.com.

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio is comprised of two approved products and multiple clinical and preclinical product candidates. Approved products include Naglazyme(R) (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin, and Aldurazyme(R) (laronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation. Investigational product candidates include Phenoptin(TM) (sapropterin dihydrochloride), a Phase 3 product candidate for the treatment of phenylketonuria (PKU), and 6R-BH4 for cardiovascular indications, which is currently in Phase 2 clinical development for the treatment of poorly controlled hypertension. For additional information, please visit www.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

BioMarin Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: financial

projections, including revenue expectations for Aldurazyme and Naglazyme; the development and commercialization of Naglazyme and Phenoptin; and actions by regulatory and governmental 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: possible delays in launching Naglazyme in the E.U. and slow market penetration in the U.S. and E.U.; actions by governmental authorities related to the reimbursement of Naglazyme; the content and timing of decisions by the FDA and European Commission and other regulatory authorities concerning Naglazyme and Phenoptin; issues or complications associated with post-marketing commitments; the results of current and future clinical trials of Phenoptin; 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 2004 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.

NOTE: Naglazyme(R) is a registered trademark of BioMarin Pharmaceutical Inc.

Aldurazyme(R) is a registered trademark of BioMarin/Genzyme LLC.

Contact: Investors and Media, Eugenia Shen of BioMarin Pharmaceutical Inc., +1-415-506-6570.

FCMN Contact: eshen@bmrn.com

SOURCE: BioMarin Pharmaceutical Inc.

CONTACT: Investors and Media, Eugenia Shen of BioMarin Pharmaceutical Inc., +1-415-506-6570

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