

# BioMarin Announces Program for ERT for Treatment of MPS IVA - Morquio A Syndrome

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BioMarin Pharmaceutical Inc. (Nasdaq and SWX: BMRN) today announced its program for its third enzyme replacement therapy (ERT) for the treatment of mucopolysaccharidosis IVA (MPS IVA), or Morquio A Syndrome. BioMarin plans to initiate a Phase 1/2 clinical trial in the first quarter of 2009.

"With two MPS drugs on the market, we plan to leverage our clinical, manufacturing and regulatory expertise to efficiently develop a treatment for Morquio patients," said Emil Kakkis, M.D., Ph.D., Chief Medical Officer of BioMarin. "Our planned program includes a clinical assessment study to measure the depth and breadth of disease as well as a separate GALNS Phase 1/2 study in Morquio Type A patients. Preliminary studies are promising and indicate that our drug candidate binds naturally to bone matrix and can adequately reach the growth cartilage after IV infusion. The skeletal system disease is a primary concern in the treatment of this disease."

The company has successfully developed and manufactures two FDA-approved enzyme replacement therapies for the treatment of MPS I and MPS VI. Naglazyme(R) (galsulfase) MPS VI is wholly developed and commercialized by BioMarin. Aldurazyme(R) (Iaronidase) for MPS I is manufactured by BioMarin and marketed by Genzyme Corporation.

Additional details of the MPS IVA program, along with an overview of BioMarin's product portfolio, advancements in the research and development pipeline and other ongoing programs will be provided today at BioMarin's R&D Day program in New York City. Interested parties may access a live audio webcast of the presentation via the investor section of the BioMarin website, <http://www.bmrn.com/>. A replay of the presentation will be archived on the site for at least one week following the presentation. For general inquiries please email [Morquio@bmrn.com](mailto:Morquio@bmrn.com)

## About MPS IVA

Mucopolysaccharidosis IVA (MPS IVA, also known as Morquio A Syndrome) is a disease characterized by deficient activity of N-acetylgalactosamine-6- sulfatase (GALNS) causing excessive lysosomal storage of keratan sulfate (KS). This excessive storage causes a systemic skeletal dysplasia, short stature, and joint abnormalities, which limit mobility and endurance. Malformation of the thorax impairs respiratory function, and odontoid hypoplasia and ligamentous laxity cause cervical spinal instability and potentially cord compression. Other symptoms may include hearing loss, corneal clouding, and heart valvular disease. Initial symptoms often become evident in the first five years of life. Depending on severity of the disease, age of diagnosis will vary.

The rate of incidence of MPS IVA is as yet unconfirmed, but estimates vary between 1 in 200,000 live births to 1 in 300,000 live births. Approximately 370 patients worldwide are currently registered in The International Morquio Organization (IMO) survey. An estimated 1,100 patients in the developed world have MPS VI and an estimated 3,000 patients in the developed world have MPS I.

## About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises three 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; Aldurazyme(R) (Iaronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; and Kuvan(R) (sapropterin dihydrochloride) Tablets, a product for the treatment of phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany. Other product candidates include 6R-BH4 for cardiovascular indications, which is currently in Phase 2 clinical development for the treatment of peripheral arterial disease and sickle cell disease, and PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase 1 clinical development for the treatment of PKU. For additional information, please visit <http://www.BMRN.com>. Information on BioMarin's website is not incorporated by reference into this press release.

## Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin

Pharmaceutical Inc., including, without limitation, statements about: the development of its program for MPS IVA, and expectations regarding filings with regulatory agencies. 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 results of current and planned clinical trials related to the enzyme replacement therapy for MPS IVA; the content and timing of decisions by the U.S. Food and Drug Administration and other regulatory agencies, particularly with respect to the enzyme replacement therapy for MPS IVA, and those factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption "Risk Factors" in BioMarin's 2007 Annual Report on Form 10-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(R), Naglazyme(R) and Kuvan(R) are a registered trademarks of BioMarin Pharmaceutical Inc.

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

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