BioMarin Announces Initiation of Clinical Assessment Program for Morquio A Syndrome

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BioMarin Pharmaceutical Inc. announced today the initiation of the Morquio Clinical Assessment Program (MorCAP) for patients with the lysosomal storage disease Mucopolysaccharidosis Type IVA (MPS IVA), or Morquio A Syndrome. MorCAP is designed to augment available data on the disease by measuring endurance and respiratory function and other parameters in affected patients. BioMarin expects to follow the MorCAP program with a Phase 1b clinical trial of enzyme replacement therapy beginning in the first quarter of 2009. The primary objectives of the Phase 1b study will be to evaluate safety and to establish the optimal dose of enzyme based on pharmacokinetic and pharmacodynamic parameters.

"After successfully advancing two enzyme replacement therapies in approximately five years each from IND filing to FDA approval, we plan to leverage our clinical, manufacturing and regulatory expertise to develop a treatment for Morquio A syndrome," said Emil Kakkis, M.D., Ph.D., Chief Medical Officer of BioMarin. "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. We recently have also shown that GALNS can reduce keratan sulfate storage in Morquio chondrocytes. This is important as the skeletal system is a primary concern in the treatment of this disease."

"We are excited to be the first site to enroll patients in the BioMarin Clinical Assessment Program for MPS IVA patients. This study is crucial to developing a deeper understanding of the clinical outcomes for this rare disorder, which will help lead to better disease management and therapy options, said Barbara Burton, M.D., Director, MPS/ML Treatment Program, Children's Memorial Hospital."

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

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 as well as macrophage storage in the lung likely impairs respiratory function and contributes to sinopulmonary infections. Odontoid hypoplasia and ligamentous laxity can commonly cause cervical spinal instability and potentially spinal cord compression. Other symptoms may include recurrent infections, 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. Many patients end up wheelchair dependent in their second decade of life and undergo numerous surgeries to manage their disease.

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 400 patients worldwide are currently registered in a public registry, based on their publications. The prevalence of patients with MPS IVA appears substantially higher than that with MPS VI based on published reports.

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) (laronidase) for mucopolysaccharidosis I (MPS I), a product which was 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 particularly the timing and conuct of clinical trials related thereto, 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 pre-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, EMEA 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.

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