BioMarin's Clinical Trial Application for GALNS for Morquio A Syndrome Accepted by the MHRA

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BioMarin Pharmaceutical Inc. announced today that its application for clinical trial authorization (CTA) for BMN 110 or N-acetylgalactosamine 6-sulfatase (GALNS), intended for the treatment of the lysosomal storage disorder Mucopolysaccharidosis Type IVA (MPS IVA), or Morquio A Syndrome, has been accepted by the United Kingdom Medicines and Healthcare Products Regulatory Agency (MHRA). BioMarin expects to initiate a Phase 1/2 clinical trial in the next few weeks.

"There are a significant number of untreated Morquio patients in clinics around the world who are very anxious to receive a new treatment option. We are moving forward expeditiously with our GALNS program in the hope of bringing those patients the much-needed treatment they deserve. BioMarin has successfully advanced two enzyme replacement therapies from IND filing to FDA approval in approximately five years each and we plan to leverage our clinical, manufacturing and regulatory expertise to develop this treatment for Morquio A syndrome," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "Enrollment in the MorCAP study initiated last November is continuing and we continue to add additional sites. We expect that this survey study will yield valuable information on Morquio patients and will aid in our scientific understanding of the disorder as we further pursue this program."

The Phase 1/2 study is designed as an open-label, within-patient dose escalation trial in approximately 20 patients followed by a treatment continuation phase. During the dose escalation phase of the study, subjects will receive weekly intravenous infusions of BMN 110 in 3 consecutive 12-week dosing intervals. The objectives of the Phase 1/2 study will be to evaluate safety, pharmacokinetics, pharacodynamics and to identify the optimal dose of GALNS for future studies.

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 disorder 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 dysfunction in the lung likely impairs respiratory function and contributes to sinopulmonary infections. Odontoid dysplasia 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 disorder, age of diagnosis will vary. Many patients become wheelchair dependent in their second decade of life and undergo numerous surgeries to alleviate life-threatening conditions caused by the underlying enzyme deficiency.

The incidence estimates for MPS IVA vary widely, between 1 in 200,000 live births to 1 in 300,000 live births. Approximately 400 patients worldwide have been identified and tracked through an independent registry. Based on the number of identified patients to date, the prevalence of patients with MPS IVA appears similar to that with 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) (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 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

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 2008 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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