Patient Enrollment Complete for Phase I/II Clinical Trial for GALNS for Morquio A Syndrome

PRNewswire-FirstCall
NOVATO, Calif.

BioMarin Pharmaceutical Inc. announced today that patient enrollment has been completed for the Phase I/II clinical trial 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. The company expects to report initial results in the first half of 2010.

"The efficient enrollment of twenty patients is a critical milestone for the MPS IVA program and demonstrates both our commitment to this program and the support and enthusiasm of the MPS IVA patient community. Data generated from this study will be valuable in demonstrating safety and could be instrumental in designing a successful Phase III trial. Assessments from the Phase I/II study such as plasma and urine keratan sulfate levels, pulmonary function and walk tests will be helpful in determining optimal Phase III endpoints," said Henry Fuchs, M.D., Chief Medical Officer of BioMarin. "We appreciate the collaboration of the Morquio community in this important effort, and we hope to develop this new treatment as expeditiously as possible."

Christian Hendriksz, MBChB, MSc, FRCPCH, MRCP, Consultant in Metabolic Disorders, Birmingham Children's Hospital, UK, added, "Morquio is a serious and debilitating disease in which accumulation of keratan sulfate results in impaired breathing and walking, recurrent infections, impaired bone and joint function, dysmorphology and an overall impaired quality of life. The rapid accrual of patients for the Phase I/II study speaks to the magnitude of the unmet need for this disease."

The Phase I/II study is designed as an open-label, within-patient dose escalation trial in 20 patients followed by a treatment continuation period. During the dose escalation phase of the study, subjects will receive weekly intravenous infusions of BMN-110 in three consecutive 12-week dosing intervals. The objectives of the Phase I/II study will be to evaluate safety, pharmacokinetics, pharmacodynamics and to identify the optimal dose of GALNS for future studies. BioMarin plans to provide an extension study in which all patients in the Phase I/II study will be eligible to participate.

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 (galsulfase) for MPS VI is wholly developed and commercialized by BioMarin. Aldurazyme (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 one in 200,000 live births to one in 300,000 live births. Approximately 400 patients worldwide have been identified and tracked through the International Morquio Organization (IMO) survey. There are already more MPS IVA patients identified through this registry than there are MPS VI patients being treated with Naglazyme worldwide. 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 (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme (laronidase) for

mucopolysaccharidosis I (MPS I), a product that was developed through a 50/50 joint venture with Genzyme Corporation; and Kuvan (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 I 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 conduct 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 clinical and 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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