BioMarin Initiates Pivotal Phase 3 Trial for GALNS for the Treatment of MPS IVA

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NOVATO, Calif., Feb. 1, 2011 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) announced today that it has initiated a pivotal Phase 3 trial for N-acetylgalactosamine 6-sulfatase (GALNS or BMN 110), intended for the treatment of the lysosomal storage disorder Mucopolysaccharidosis Type IVA (MPS IVA), also called Morquio A Syndrome.

"In under two years, we have progressed the GALNS program from Clinical Trial Application to initiation of the Phase 3 trial. We have received FDA feedback and have finalized the design of the Phase 3 pivotal trial," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "The study will be conducted at approximately 40 centers worldwide including Brazil, Japan, Taiwan, most Western European countries, Canada and the U.S. The trial is expected to enroll approximately 160 subjects and will be the largest enzyme replacement therapy trial conducted. There are no therapeutic options for MPS IVA patients who have a high unmet medical need. Initiation of this well-designed pivotal study is an important milestone for both the company and the MPS IVA community."

The Phase 3 trial is a randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of GALNS in patients with MPS IVA. The study will explore doses of two mg/kg/week and two mg/kg/every other week for a treatment period of 24 weeks. The primary endpoint is the six-minute walk test, and the secondary endpoints are the three-minute stair climb test and urine keratan sulfate concentration.

About MPS IV

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 potential 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 and varies among different populations but estimates vary between 1 in 200,000 live births and 1 in 250,000 live births. There are several studies that have documented the incidence as high as 1 in 76,000 live births in Northern Ireland. The estimated prevalence is between 1,000 and 1,500 patients in the U.S., EU and Japan and between 1,500 to 2,000 patients in the rest of the world for a total of 2,500 to 3,500 patients. Over 1,000 MPS IVA patients worldwide have been identified through The International Morquio Organization (IMO) survey and the BioMarin MorCAP registry program.

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises four approved products and multiple clinical and pre-clinical 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 which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; Kuvan® (sapropterin dihydrochloride) Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; and Firdapse™ (amifampridine phosphate), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Other product candidates include GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase III clinical development for the treatment of MPS IVA, PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase II clinical development for the treatment of PKU, BMN 701, a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), which is currently in Phase I/II clinical development for the treatment of Pompe disease, and BMN 673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase I/II clinical development for the treatment of genetically-defined cancers. 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, expectations regarding other clinical and preclinical programs, and the potential market for GALNS, if approved. 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 BioMarin's development programs and particularly 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, the actual number of MPS IVA patients in the developed world; 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 2009 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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