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Press Release

BioMarin Initiates Phase 1/2 Trial for BMN 701 for the Treatment of Pompe Disease

NOVATO, Calif., Jan. 19, 2011 /PRNewswire via COMTEX/ --

BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) announced today that it has initiated a Phase 1/2 trial for BMN 701, a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA) in development for the treatment of Pompe disease.

"We have a strong track record of quickly developing enzyme replacement therapies for unmet medical needs and expect to leverage our clinical and regulatory experience and manufacturing know-how in the development of BMN 701," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "There is a significant amount of interest in the medical community for a more effective treatment option for late-onset Pompe disease, and we believe, based on in vitro and in vivo nonclinical studies, that using our proprietary Glycosylation Independent Lysosomal Targeting, or GILT technology, BMN 701 has the potential to deliver more enzyme to lysosomes compared to traditional mannose-6-phosphate targeted approaches."

The Phase 1/2 trial is an open-label study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamic and clinical activity of BMN 701 administered as an intravenous infusion every two weeks at doses of 5 mg/kg, 10 mg/kg and 20 mg/kg. The company expects to enroll approximately 30 patients between the ages of 13 and 65 years old with late-onset Pompe disease for a treatment period of 24 weeks.

About Pompe Disease

Pompe disease, a lysosomal storage disorder, is a progressive degenerative disease of skeletal muscle including respiratory muscles such as diaphragm, and of heart muscle in infant-onset patients. It is caused by a deficiency in the lysosomal enzyme acid alpha glucosidase which leads to the accumulation of glycogen in muscle cell lysosomes and results in cell death. The incidence is one in 40,000 births. There are two main forms of Pompe disease: adult onset with an incidence of one in 57,000 births and infantile onset with an incidence of one in 138,000 births. Prognosis with standard of care is stabilization of the disease or minor improvements for the majority of adult onset patients.

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(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 BioMarin developed through a 50/50 joint venture with Genzyme Corporation; Kuvan(R) (sapropterin dihydrochloride) Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; and Firdapse(TM) (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 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) in Phase 1/2 development for the treatment of Pompe disease, and BMN 673, a poly ADP-ribose polymerase (PARP) inhibitor in Phase 1/2 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 expectations related to regulatory actions on BMN 701 and the development and efficacy of BMN 701. 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 content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities, results and timing of current and planned preclinical studies and clinical trials related to such product; our ability to successfully manufacture the product; 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, and the factors contained in BioMarin's reports on Form 10-Q. 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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