BioMarin Initiates Phase 1 Clinical Study of BMN 195 for Duchenne Muscular Dystrophy

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BioMarin Pharmaceutical Inc. announced today that the first subject has initiated treatment in the Phase 1 clinical study of BMN 195, a small molecule utrophin upregulator, for the treatment of Duchenne muscular dystrohpy (DMD). Initial top-line results are expected in the third quarter of 2010.

"Duchenne muscular dystrophy represents a serious unmet medical need affecting approximately 40,000 patients in the developed world, and we are excited to advance our program into the clinic in hopes of providing the first therapeutic option to treat this disease," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "BMN 195 has been shown to upregulate utrophin levels in human muscle cells, as a means of augmenting muscle function. In mice with mutations in the normal dystrophin gene, BMN 195 has been shown to improve strength. Therefore, BMN 195 may have the potential to treat the entire spectrum of DMD patients, regardless of the type of genetic abnormality."

The Phase 1 clinical trial is a single-center, double-blind, placebo-controlled, single-dose escalation study followed by a multiple-dose escalation study of BMN 195 administered orally in healthy volunteers. The primary objective is to assess the safety, tolerability and pharmacokinetics of BMN 195 in healthy volunteers, and enable subsequent studies in patients with DMD.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy is a fatal neuromuscular disorder that affects 1 in 3,500 boys with an estimated patient population of over 40,000 in the developed world.

DMD is caused by a genetic defect that results in DMD patients lacking an important protein called dystrophin, which is crucial to maintaining muscle integrity and function. The absence of dystrophin results in extensive muscle wasting in all voluntary muscles as well as the heart and breathing muscles and causes severe restriction in the mobility of DMD patients by their early teens and is ultimately fatal, generally in their twenties. Currently there is no cure for DMD. Corticosteroid treatment is the only frontline therapy and acts to only delay the progression of the disease.

About BMN 195

BMN 195 is a proprietary, orally available small molecule with a novel mechanism of action for DMD. BMN 195 acts by increasing expression of utrophin, an endogenous protein that is functionally similar to dystrophin. The goal of therapy would be to preserve muscle function and prevent the inexorable decline in strength seen in DMD patients. BioMarin believes the primary advantage of BMN 195 is that it offers the potential to treat the entire DMD patient population, regardless of the mutation the patient carries.

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 3,4-diaminopyridine (amifampridine phosphate), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Other product candidates include PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase II clinical development for the treatment of PKU and GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase I/II clinical development for the treatment of MPS IVA. 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 product candidate

BMN 195, and expectations related to clinical trials of BMN 195. 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 trials related to BMN 195; the content and timing of decisions by the U.S. Food and Drug Administration and other regulatory agencies, particularly with respect to BMN 195, 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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