FDA Grants Orphan Drug Designation for 3,4-DAP for LEMS

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BioMarin Pharmaceutical Inc. announced today that the Food and Drug Administration (FDA) has granted orphan drug designation for 3,4-diaminopyridine (3,4-DAP), amifampridine phosphate, for the rare autoimmune disease Lambert Eaton Myasthenic Syndrome (LEMS). 3,4-DAP has previously received orphan drug designation in the E.U. Also, in October 2009, the Committee for Medicinal Products for Human Use of the European Medicines Evaluations Agency adopted a positive opinion recommending approval of amifampridine phosphate for LEMS. If approved by the European Commission, amifampridine phosphate will be the first approved treatment for LEMS, thereby conferring orphan drug protection and providing ten years of market exclusivity in Europe.

"With its experience in the development and commercialization of orphan drugs, BioMarin is well-positioned to seek U.S. registration of amifampridine phosphate for LEMS, a serious and debilitating autoimmune disease often associated with small cell lung cancer," said Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. "We look forward to meeting with the FDA in early 2010 to determine the necessary regulatory path for amifampridine phosphate in the U.S. We are also preparing to launch the product in Europe in the first quarter of 2010, and will also evaluate the best development strategy for amifampridine phosphate in other indications in the U.S. and Europe."

About LEMS

Lambert Eaton Myasthenic Syndrome (LEMS) is a rare autoimmune disease with the primary symptoms of muscle weakness. Muscle weakness in LEMS is caused by autoantibodies to voltage gated calcium channels leading to a reduction in the amount of acetylcholine released from nerve terminals. The prevalence of LEMS is estimated at four to ten per million, or approximately 2,000 to 5,000 patients in the E.U. and 1,200 to 3,100 patients in the U.S. Approximately 50 percent of LEMS patients diagnosed have small cell lung cancer.

Patients with LEMS typically present with fatigue, muscle pain and stiffness. The weakness is generally more marked in the proximal muscles particularly of the legs and trunk. Other problems include reduced reflexes, drooping of the eyelids, facial weakness and problems with swallowing. Patients often report a dry mouth, impotence, constipation and feelings of light headedness on standing. On occasion these problems can be life threatening when the weakness involves respiratory muscles. A diagnosis of LEMS is generally made on the basis of clinical symptoms, electromyographic testing and the presence of autoantibodies against voltage gated calcium channels.

Current treatment of LEMS can consist of strategies directed at the underlying malignancy if one is present. Unfortunately, therapy of small cell lung cancer is limited and outcomes are generally poor. Immunosuppressive agents have been tried but success is limited by toxicity, and difficulty administering the regimens. A mainstay of therapy has been 3,4-DAP but its use in practice has been limited by the drug's availability. This problem will be addressed by the introduction of BioMarin's product.

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 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; and Kuvan® (sapropterin dihydrochloride) Tablets, for 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 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 expectations of the development and

potential approval of Huxley's 3,4-Diaminopyridine product for the treatment of LEMS. 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, particularly the pending decision by the European Commission on the Marketing Authorization Application for such product, our success in the commercialization of such product, if approved; 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 2008 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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