BioMarin Submits Drisapersen MAA to EMA for the Treatment of Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping

SAN RAFAEL, Calif., June 8, 2015 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today the submission of a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for drisapersen, an investigational antisense oligonucleotide drug candidate for the treatment of the largest subset of Duchenne muscular dystrophy (DMD) amenable to single exon skipping. DMD is the most common fatal genetic disorder diagnosed in childhood, affecting approximately 1 in every 3,500 live male births with about 20,000 new cases diagnosed globally each year. In Europe, it is estimated there are 23,000 boys with Duchenne Muscular Dystrophy, and approximately 3,000 of those would be candidates for drisapersen. In BioMarin's commercial territories, approximately 85 percent of Duchenne patients are located outside of the United States, including Western Europe, Middle East, Eastern Europe, Latin America and Japan. Western Europe has the largest patient population among those areas, exceeding the United States by around 30 percent.

Drisapersen induces the skipping of dystrophin exon 51, potentially providing a therapeutic benefit to DMD patients for whom skipping of exon 51 restores the proper dystrophin reading frame, corresponding to approximately 13% of DMD patients. The company recently submitted a New Drug Application (NDA) to the United States Food and Drug Administration (FDA) for drisapersen in April 2015.

"The submission of this application to the EMA represents an important achievement in BioMarin's efforts to bring a meaningful therapeutic option to patients and families around the world with Duchenne muscular dystrophy. We were able to reach this point because of the extraordinary effort of the employees at BioMarin, the investigators for the clinical trials and most important, the boys and their families who participated in the clinical trials," said Camilla V. Simpson, Global Head of Regulatory Affairs, Pharmacovigilance. "BioMarin has a track record of efficiently developing therapies in rare genetic diseases, and we are pleased that we have submitted this MAA ahead of the expected timeline. We look forward to working with the EMA in the coming months with the goal of bringing this therapy to patients in need."

"We applaud BioMarin's commitment to develop a therapy for Duchenne muscular dystrophy," said Elizabeth Vroom, Chair of United Parent Projects Muscular Dystrophy (UPPMD). "The community has been supporting research and development of treatments, and we are pleased that drisapersen has been submitted for EMA review. We are hopeful that this therapy will lead not only to an approved therapy, but will further scientific advances and the development of other treatments for boys with Duchenne."

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy is a severely debilitating childhood neuromuscular disease that affects up to 1 in 3,500 live male births. In BioMarin's commercial territories, approximately 85 percent of patients live outside of the United States. This rare disease is caused by mutations in the dystrophin gene, resulting in the absence or defect of the dystrophin protein, which is important in connecting the cytoskeleton of muscle fibers to the extracellular matrix. As a result, patients suffer from progressive loss of muscle strength, often rendering them wheelchair-bound before the age of 12 years. Respiratory and cardiac muscle can also be affected by the disease and most patients die in early adulthood due to respiratory and cardiac failure. Because the Duchenne gene is found on the X-chromosome, it primarily affects boys.

About Drisapersen and Exon Skipping

In Duchenne muscular dystrophy, mutations in the dystrophin gene lead to the absence of dystrophin protein, resulting in the most severe form of dystrophin deficient muscular dystrophy. Drisapersen is an antisense oligonucleotide that induces exon skipping to provide a molecular patch for dystrophin transcripts produced by certain mutated dystrophin genes. Exons are the parts of a gene that contain the instructions for generating a protein. In applicable cases, skipping an exon near the mutation allows for the production of a truncated but functional dystrophin protein.

The drisapersen clinical dataset includes data from more than 300 patients in three randomized, placebo-controlled, efficacy trials and two ongoing long-term extension studies, in which some boys have been treated for approximately 3.4 years.

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises five approved products and multiple clinical and pre-clinical product candidates. Approved products include Vimizim® (elosulfase alfa) for MPS IVA, a product wholly developed and commercialized by BioMarin; Naglazyme® (galsulfase) for MPS VI, a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for MPS I, a product which BioMarin developed through a 50/50 joint venture with Genzyme Corporation; Kuvan® (sapropterin dihydrochloride) Powder for Oral Solution and Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany and Firdapse® (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Product candidates include drisapersen, an exon skipping oligonucleotide, for which a marketing application has been submitted to FDA and EMA for the treatment of patients with Duchenne muscular dystrophy (DMD) with mutations in the dystrophin gene that are amenable to treatment with exon 51 skipping, pegvaliase (PEGylated recombinant phenylalanine ammonia lyase, formerly referred to as BMN 165 or PEG PAL), which is currently in Phase 3 clinical development for the treatment of PKU, talazoparib (formerly referred to as BMN 673), a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer, reveglucosidase alfa (formerly referred to as BMN 701), a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), which is currently in Phase 3 clinical development for the treatment of Pompe disease, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 2 clinical development for the treatment of achondroplasia, BMN 044, BMN 045 and BMN 053, exon skipping oligonucleotides, which are currently in Phase 2 clinical development for the treatment of Duchenne muscular dystrophy (exons 44, 45 and 53), cerliponase alfa (formerly referred to as BMN 190), a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of CLN2 disorder, a form of Batten disease, which is currently in Phase 1, BMN 270, an AAV-factor VIII vector, for the treatment of hemophilia A and BMN 250, a novel fusion of alpha-N-acetyglucosaminidase (NAGLU) with a peptide derived from insulin-like growth factor 2 (IGF2), for the treatment of MPS IIIB.

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: expectations regarding the MAA submission for drisapersen with the EMA and the FDA; the potential outcome of the review of such filings; and the possible approval of drisapersen. 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: results and timing of current and planned clinical trials of drisapersen; the content and timing of decisions by the FDA, the EMA and other regulatory authorities concerning drisapersen; 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 2014 Annual Report on Form 10-K, as amended, and the factors contained in BioMarin's reports on Form 8-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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