BioMarin Completes Rolling NDA Submission to FDA for Drisapersen for Treatment of Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping

SAN RAFAEL, Calif., April 27, 2015 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced completion of the rolling submission of a New Drug Application (NDA) to the United States Food and Drug Administration (FDA) for drisapersen, an investigational exon-skipping drug candidate for the treatment of the largest genetically defined subset of Duchenne muscular dystrophy (DMD). 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. 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 intends to also submit an application for registration in the European Union in summer 2015.

"We believe drisapersen may offer a meaningful benefit to boys living with DMD whose mutations are amenable to exon 51 skipping. The totality of data on drisapersen contains three randomized, placebo-controlled, efficacy trials and two long term extension studies, which include some boys treated for approximately 3.4 years," said Camilla V. Simpson, Global Head of Regulatory Affairs, Pharmacovigilance. "With this application, BioMarin continues in its long-standing tradition of developing important therapies for those who are most in need. The submission of the NDA represents a significant milestone for BioMarin, and we appreciate the strong, collaborative effort of many hard working employees, investigators, patients and their families. We look forward to working with the U.S. Regulatory Authorities to thoroughly understand the data generated for this heterogenous and critically ill patient population and hopefully to bring this treatment to patients expeditiously."

Drisapersen has been granted Orphan and Fast Track status, as well as Breakthrough Therapy designation by the FDA.

DMD is caused by a mutation in the gene that encodes for dystrophin, a protein that is important in connecting the cytoskeleton of muscle fibers to the extracellular matrix. Its deficiency in DMD leads to progressive muscle weakness, loss of ambulation in early adolescence, and typically death due to pulmonary or cardiac insufficiency in the late twenties. Because the Duchenne gene is found on the X-chromosome, it primarily affects boys; however, it occurs across all races and cultures. There is currently no approved therapy in the United States for DMD.

"This is a first for the Duchenne community, and we are filled with hope that there could be a treatment for Duchenne in the United States," said Debra Miller, co-founder and CEO of CureDuchenne. "CureDuchenne has been supporting the development of drisapersen for more than a decade, and we are delighted that BioMarin has reached this important stage. We salute the researchers who have been working so hard, and we share their determination to find a cure for Duchenne."

About Drisapersen

Duchenne muscular dystrophy (DMD) is a severely debilitating childhood neuromuscular disease that affects up to 1 in 3,500 live male births. This rare disease is caused by mutations in the dystrophin gene, resulting in the absence or defect of the dystrophin protein. 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.

About Exon Skipping

Exons are the parts of a gene that contain the instructions for generating a protein. In DMD, mutations in the dystrophin gene lead to the absence of dystrophin protein, resulting in the most severe form of muscular dystrophy. In applicable cases, skipping an exon near the mutation allows for the production of a truncated but functional dystrophin protein.

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 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 rolling NDA submission for drisapersen with the FDA and the pending submission to the European Medicines Agency (EMA); the potential outcome of the review of such filings; and the possible approval of such product candidates. 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 its product candidates; the content and timing of decisions by the FDA, the EMA and other regulatory authorities concerning its product candidates; 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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