BioMarin Receives Rare Pediatric Disease Designation From FDA for Drisapersen for the Potential Treatment of Duchenne Muscular Dystrophy

SAN RAFAEL, Calif., Aug. 19, 2015 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that the U.S. Food and Drug Administration (FDA) has granted rare pediatric disease designation for drisapersen, a potential treatment for patients with Duchenne Muscular Dystrophy (DMD) who are amenable to exon 51 skipping treatment. The FDA has previously granted drisapersen Orphan and Fast Track Status, Breakthrough Therapy Designation, as well as Priority Review status, which is designated to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists.

"BioMarin has a track record of efficiently developing therapies to treat rare and ultra-rare genetic diseases and we are committed to bringing drisapersen to children who desperately need an approved treatment option in the U.S. We are pleased that the FDA's Office of Orphan Products Development has granted drisapersen this designation," said Jean-Jacques Bienaimé, Chairman and Chief Executive Officer of BioMarin. "BioMarin was the first Company to receive a Rare Pediatric Disease Priority Review Voucher upon the approval of Vimizim®, in February of 2014. We are dedicated to developing important new therapeutics for patients, mostly children, with life threatening disorders, and this designation for drisapersen is a testament to BioMarin's deep commitment to that goal."

About Rare Pediatric Disease Designation

The FDA defines a "rare pediatric disease" as a disease that affects fewer than 200,000 individuals in the U.S. primarily aged from birth to 18 years. Under the

FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval of a new drug application (NDA) or biologics license application (BLA) for a rare pediatric disease may be eligible for a voucher which can be redeemed to obtain priority review for a subsequent marketing application for a different product. The Priority Review Voucher may be sold or transferred an unlimited number of times.

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, vosoritide (formerly referred to as 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: the FDA rare pediatric disease designation for drisapersen and the possibility of obtaining a priority review voucher as a result. 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 timing and success of the

FDA review of the pending new drug application for drisapersen; results and timing of current and planned preclinical studies and clinical trials of 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, 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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