BioMarin Sells Priority Review Voucher for $67.5 Million

SAN RAFAEL, Calif., July 30, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that it has sold the Rare Pediatric Disease Priority Review Voucher (PRV) it obtained in February of this year. The Company received the voucher under an FDA program intended to encourage the development of treatments for rare pediatric diseases. BioMarin was awarded the voucher when it received approval of VIMIZIM®, a new biological product for patients with Mucopolysaccharidosis type IVA, also known as Morquio A syndrome. BioMarin received $67.5 million from Regeneron Ireland, an indirect, wholly-owned subsidiary of Regeneron Pharmaceuticals, Inc., in exchange for the voucher.

"Leveraging the sale of the Priority Review Voucher to reinvest in products to treat rare and ultra-rare diseases makes the most sense for BioMarin given our stage of growth," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "We are very pleased that a patient population beyond BioMarin's will potentially be able to benefit from a faster drug application review process."

About the Pediatric Disease Priority Review Voucher Program

The Pediatric PRV is issued to the sponsor of a rare pediatric disease product application that entitles the holder to priority review of a single New Drug Application or Biologics License Application. The sponsor receives the voucher upon approval of the rare pediatric disease product application. Pediatric PRVs may be sold or transferred, and there is no limit on the number of times a priority review voucher can be transferred.

About Food and Drug Administration Standard Review and Priority Review Designations

Prior to approval, each drug marketed in the United States must go through a detailed FDA review process. In 1992, under the Prescription Drug User Act (PDUFA), FDA agreed to specific goals for improving the drug review time and created a two-tiered system of review times - Standard Review and Priority Review. Standard Review can be accomplished in a ten-month time frame from the time the application is filed by the FDA, which typically occurs approximately 60-days following submission of the application. A Priority Review designation is given to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. The FDA goal for reviewing a drug with Priority Review status is six months from the time the application is filed by the FDA.

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: 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) Powder for Oral Solution and Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; Firdapse® (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS); and VIMIZIM® (N-acetylgalactosamine 6-sulfatase) for the treatment of Morquio A (MPS IVA). Product candidates include: BMN 165 (PEGylated recombinant phenylalanine ammonia lyase), also referred to as PEG PAL, which is currently in Phase 3 clinical development for the treatment of PKU; 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; BMN 701, a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin like growth factor 2, 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; and BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease.

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