BioMarin Announces French ATU Granted for Vimizim(TM) for the Treatment of Morquio A Syndrome

SAN RAFAEL, Calif., Nov. 15, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that the French National Agency for Medicines and Health Products Safety (ANSM) has granted an Autorisation Temporaire d'Utilisation de cohorte (ATU cohort), or Temporary Authorization for Use, for patient sales of Vimizim for the treatment of Morquio A Syndrome. An ATU is the regulatory mechanism used by the ANSM to make non-approved drugs available to patients in France when a genuine public health need exists. This ATU allows patients with Morquio A Syndrome in France to receive treatment with Vimizim before marketing authorization for the product is granted in the European Union. Government allocations to hospitals allow payment for Vimizim for patients included in the ATU program. The company expects to book revenue on named-patient basis sales and will continue to assist patients in France wherever possible prior to full market product approval in the European Union.

"With a significant number of all Morquio A patients living throughout Europe, we are pleased to be able to provide Vimizim to patients in France under the ATU program," said Jeff Ajer, Senior Vice President, Global Commercial Operations at BioMarin. "We look forward to working closely with European regulatory authorities through the final stages of full market approval in this region."

About Morquio A Syndrome

Mucopolysaccharidosis IVA (MPS IVA, also known as Morquio A syndrome) is a disease characterized by deficient activity of Nacetylgalactosamine- 6-sulfatase (GALNS) causing excessive lysosomal storage of glycosaminoglycans such as keratan sulfate and chondroitin sulfate. This excessive storage causes a systemic skeletal dysplasia, short stature, and joint abnormalities, which limit mobility and endurance. Malformation of the chest impairs respiratory function, and looseness of joints in the neck cause spinal instability and potentially spinal cord compression. Other symptoms may include hearing loss, corneal clouding, and heart disease. Initial symptoms often become evident in the first five years of life. The disease substantially limits both the quality and length of life of those affected.

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises four 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) 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 Vimizim™ (N-acetylgalactosamine 6-sulfatase), formally referred to as GALNS, which successfully completed Phase 3 clinical development for the treatment of MPS IVA, PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), 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 1/2 clinical development for the treatment of Pompe disease, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 1 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. 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 regulatory status of Vimizim, possible sales of Vimizim under the ATU program and expectations about the commercialization of Vimizim generally. These risks and uncertainties include, among others: the ability of hospitals to use the ATU program and the funding for the ATU program generally; results and timing of current and planned preclinical studies and clinical trials of Vimizim; the content and timing of decisions by the U.S. Food and Drug Administration, the European

Commission and other regulatory authorities concerning Vimizim; 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 2012 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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