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For Immediate Release:

BioMarin's VIMIZIM® (elosulfase alfa) Approved in Australia for Treatment of Morquio A Syndrome

First and Only Treatment in Australia for Patients with this Ultra-Rare Genetic Condition

SAN RAFAEL, Calif., Dec. 8, 2014 -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced the Australian Therapeutic Goods Administration (TGA) granted approval of the registration of VIMIZIM® (elosulfase alfa) for the treatment of patients with mucopolysaccharidosis type IVA (MPS IVA), also known as Morquio A syndrome. VIMIZIM is the first treatment in Australia approved for this condition. VIMIZIM was reviewed under the Orphan Drug program.

Dr. Kaustuv Bhattacharya from The Children's Hospital at Westmead, who is currently using VIMIZIM on patients in a research trial said, "Morquio syndrome is a rare and difficult condition to have. VIMIZIM is the only definitive treatment available and offers the hope of better endurance to these patients in the future."

Morquio A syndrome is an ultra-rare, severely debilitating disease affecting an estimated 3,000 patients in the developed world. The disease occurs as a result of a deficiency of activity in an enzyme involved in glycosaminoglycan (GAG) metabolism. The pervasive and progressive accumulation of GAGs leads to significant morbidities and multisystemic clinical impairments resulting in diminished functional capacity, impaired quality-of-life and early mortality. The most common features of the disease are progressive skeletal dysplasia, the need for frequent surgical procedures related primarily to musculoskeletal or respiratory dysfunction, and significant limitations in mobility, endurance and breathing.

"Treatment for Morquio A syndrome has been lagging behind for a number of years, leaving patients with few options that could really make a difference in their daily lives," said Nicole Millis, National Manager of the MPS & Related Diseases Society, Australia. "We are grateful for BioMarin's commitment to the MPS community and for providing a therapy to these patients."

The U.S. Food and Drug Administration (FDA) approved the VIMIZIM license application for the treatment of patients with Morquio A syndrome on February 14, 2014. The therapy is also

approved in Canada and the European Union. Marketing applications have been submitted in several other countries.

“The approval of VIMIZIM in Australia underscores our relentless commitment to providing this much needed therapy to patients with Morquio A syndrome across the globe,” said Hank Fuchs, M.D., chief medical officer of BioMarin. “We will continue to leverage our expertise and dedicate our resources to advancing therapies within the MPS community, as well as other communities with ultra-rare conditions, to ensure patients get the treatments they deserve.”

About VIMIZIM
VIMIZIM® (elosulfase alfa) is a treatment for patients with Morquio A syndrome, or mucopolysaccharidosis IVA (MPS IVA). VIMIZIM is the first approved enzyme replacement therapy (ERT) designed to target the underlying cause of Morquio A Syndrome—a deficiency in the enzyme N-acetylgalactosamine-6 sulfatase (GALNS). VIMIZIM is intended to provide the exogenous enzyme GALNS that will be taken up into the lysosomes and increase the catabolism of GAGs. Morquio A syndrome is a rare, severely debilitating and progressive disease that previously had no approved, standard-of-care treatment other than supportive care.

Minimum Product Information
VIMIZIM® elosulfase alfa (rch) 1 mg/mL Concentrate for Solution for Infusion is **indicated** for the treatment of mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome). It is **contraindicated** in MPS IVA patients who experience severe or life-threatening hypersensitivity to the active substance or to any of the excipients, if hypersensitivity is not controllable**.** Clinically significant **precautions** include anaphylaxis and severe allergic reactions**;** spinal or cervical cord compression, which is a known and serious complication of MPS IVA; and acute respiratory complications in patients with acute febrile or respiratory illness at the time of VIMIZIM infusion. The most **common adverse effects** are Infusion Reactions (IRs) with symptoms of headache, nausea, vomiting, pyrexia, chills, and abdominal pain. The most serious Infusion Reactions include anaphylaxis and hypersensitivity. See full Product Information (PI) for details. The recommended **dosage** for VIMIZIM is 2 mg/kg administered once a week. The total volume of the infusion should be delivered over approximately 4 hours. One vial contains 5 mg/5mL for dilution with 0.9% sodium chloride for Intravenous Infusion.

Pharmaceutical Benefits Scheme (PBS): This product is not listed on the PBS. Please see full Product Information.

About Morquio A Syndrome
Morquio A syndrome, or Mucopolysaccharidosis IVA (MPS IVA) is a disease in which people are missing an enzyme essential in the breakdown and removal of the glycosaminoglycans (GAGs) called keratan sulfate (KS) and chondroitin-6-sulfate (C6S). The incompletely broken down GAGs remain stored in cells in the body causing progressive damage. This excessive storage causes systemic skeletal dysplasia, short stature, and joint abnormalities, limiting mobility and endurance. Malformation of the chest impairs respiratory function, and looseness of joints in the neck causing 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.

The rate of incidence of Morquio A syndrome is as yet unconfirmed and varies among different populations, and estimates vary between 1 in 200,000 live births and 1 in 450,000 live births.

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 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, 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, 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 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 marketing application filing for Vimizim with the Australian Therapeutic Goods Administration; and the marketing and commercialization of Vimizim in Australia. 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; any further actions by the Australian Therapeutic Goods Administration; the outcome of pricing and reimbursement negotiations with relevant authorities in Australia; 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 2013 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.