BioMarin Announces FDA Advisory Committee to Review Drisapersen for Treatment of Patients With Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping

Advisory Committee Meeting Scheduled for November 24, 2015

SAN RAFAEL, Calif., Oct. 15, 2015 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that the Peripheral and Central Nervous System Drugs Advisory Committee of the U.S. Food and Drug Administration (FDA) will review the New Drug Application (NDA) for drisapersen. The FDA is currently reviewing drisapersen for the treatment of patients with Duchenne muscular dystrophy amenable to exon 51 skipping. The advisory committee will review drisapersen data included in a new drug application (NDA) during a meeting on November 24, 2015.

"Duchenne muscular dystrophy is a deadly, progressive muscle disorder with limited treatment options that affects mostly boys and young men. We welcome the necessary regulatory review processes to move treatment for this community beyond supportive care to a therapy that addresses the underlying cause of the disease," said Camilla V. Simpson, Global Head of Regulatory Affairs, Pharmacovigilance. "We look forward to discussing the New Drug Application for drisapersen with the advisory committee with the goal of bringing this much-needed treatment one step closer to patients who have a specific type of Duchenne muscular dystrophy."

Drisapersen is an investigational antisense oligonucleotide drug candidate for the treatment of the largest subset of Duchenne muscular dystrophy patients amenable to single exon skipping. In the U.S., it is estimated there are approximately 2,000 patients who might benefit from treatment with drisapersen.

The Prescription Drug User Fee Act (PDUFA) action date for completion of FDA review of the drisapersen NDA is December 27, 2015. The FDA has granted drisapersen Priority Review status, which is designated to drugs that offer major advances in treatment, or provide a treatment where no adequate therapy exists. The FDA has also granted drisapersen Orphan and Fast Track status, as well as Breakthrough Therapy designation.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy is a progressive muscle disorder that leads to serious heart or respiratory-related complications by early adulthood. Primarily affecting boys, Duchenne is diagnosed in approximately 1 in every 3,500-5,000 live male births, making it the most common fatal genetic disorder diagnosed in childhood.

The condition is caused by changes, or mutations, in an essential gene necessary for muscle function. Mutations of this gene prevent the production of dystrophin, a protein which plays an important structural role in the performance of muscles. Without dystrophin, boys living with Duchenne experience progressive muscle weakness, resulting in serious medical complications and death. There is currently no FDA approved therapy designed specifically to treat Duchenne.

About Drisapersen and Exon Skipping

In Duchenne muscular dystrophy, mutations in the dystrophin gene lead to the absence of dystrophin protein, resulting in the most severe form of dystrophin deficient muscular dystrophy. Drisapersen is an antisense oligonucleotide that induces exon skipping to provide a molecular patch for dystrophin transcripts produced by certain mutated dystrophin genes. Exons are the parts of a gene that contain the instructions for making a protein. Drisapersen, an antisense

oligonucleotide, induces skipping of dystrophin exon 51, allowing for a cell's machinery to compensate for mutations in adjacent exons, thereby allowing for the production of a truncated but functional dystrophin protein.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare and ultra-rare genetic diseases. The company's portfolio consists of five commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.BMRN.com.

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 FDA's review of the drisapersen NDA, Priority Review, Orphan and Fast Track status, Breakthrough Therapy designation, outcomes of the review of such filings; and the possible approval of drisapersen. 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 drisapersen; the content and timing of decisions by the FDA, and other regulatory authorities concerning 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, 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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