BioMarin Posts Presentation From FDA Advisory Committee Meeting for Kyndrisa(TM) (drisapersen) for the Treatment of Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping

SAN RAFAEL, Calif., Nov. 24, 2015 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that the Company's presentation from the Peripheral and Central Nervous System Drugs Advisory Committee of the U.S. Food and Drug Administration (FDA)'s meeting for Kyndrisa™ (drisapersen) is now available. The Advisory Committee is meeting today to discuss the data submitted to support the New Drug Application (NDA) for Kyndrisa for the treatment of Duchenne muscular dystrophy amenable to exon 51 skipping.

Interested parties may access BioMarin's presentation from today's Advisory Committee meeting via the link below to the investor section of the BioMarin website.

http://investors.bmrn.com/common/download/download.cfm?companyid=ABEA-3W276N&fileid=863346&filekey=AEF00248-5747-4ACF-8C2F-39F5B1D7FCC3&filename=BMRN_AdCom_with_safe_harbor_112415.pdf

About Duchenne Muscular Dystrophy

Changes in the dystrophin gene (mutations) that lead to the near absence of dystrophin protein result in the most severe form of dystrophin deficient muscular dystrophy, Duchenne muscular dystrophy, also known as just Duchenne. Dystrophin protein plays an important structural role in the performance of muscles. Without dystrophin, boys living with Duchenne experience progressive muscle weakness, causing serious medical complications including serious heart or respiratory-related complications, resulting in death in early adulthood.

Primarily affecting boys, Duchenne affects approximately 1 in every 3,500-5,000 male children, making it the most common fatal genetic disorder diagnosed in childhood.

There is currently no FDA approved therapy designed specifically to treat Duchenne.

About Kyndrisa and Exon Skipping

Kyndrisa 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 generating a protein. In applicable cases, skipping an exon near the mutation allows for the production of a truncated but functional dystrophin protein.

Kyndrisa is the first and only investigational medicine designed specifically for the treatment of Duchenne that has received orphan drug, breakthrough drug, fast track and priority review status by the FDA. The Kyndrisa clinical development program is the largest ever submitted to the FDA for the condition and includes more than 300 Duchenne patients and multiple randomized placebo-controlled studies.

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.

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