FDA Issues Complete Response Letter for KyndrisaTM for Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping

Marketing Application in Europe Remains Under Review

SAN RAFAEL, Calif., Jan. 14, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that the U.S. Food and Drug Administration (FDA) issued a Complete Response letter to the Company's New Drug Application (NDA) for Kyndrisa™ (drisapersen) for the treatment of Duchenne muscular dystrophy (Duchenne) amenable to exon 51 skipping.

The FDA issues Complete Response letters to indicate that the review cycle for an application is complete and that the application is not ready for approval in its present form. FDA has concluded that the standard of substantial evidence of effectiveness has not been met. BioMarin is reviewing the Complete Response Letter and will work with the FDA to determine the appropriate next steps regarding this application.

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.

The ongoing Kyndrisa extension studies will continue, as will the ongoing clinical trials for other exon-skipping oligonucleotides, BMN 044, BMN 045 and BMN 053, while BioMarin is exploring next steps for this application. Patients currently receiving Kyndrisa, BMN 044, BMN 045 and BMN 053 will remain on therapy.

Kyndrisa Marketing Authorization Application Remains Under Regulatory Review in Europe

An application for marketing approval of Kyndrisa is also under review in the European Union. BioMarin anticipates that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) will provide an opinion for the company's Marketing Authorization Application (MAA) for Kyndrisa for the treatment of Duchenne muscular dystrophy (Duchenne) amenable to exon 51 skipping in the first half of 2016. If the CHMP opinion is positive, the MAA will be referred to the European Commission (EC). If the MAA is approved by the EC, BioMarin would receive marketing authorization for Kyndrisa in all EU Member States. The EC is expected to render a final decision for Kyndrisa in the second half of 2016.

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.

About Duchenne Muscular Dystrophy

Duchene muscular dystrophy is an x-linked genetic disorder that affects mostly boys. In Duchenne, boys begin to show signs of muscle weakness as early as two to five years of age. The disease gradually weakens the skeletal or voluntary muscles in the arms, legs and trunk. Due to progressive muscle weakness, Duchenne patients are often wheelchair bound between the ages of seven and 13 years old. At a later stage, the boys' respiratory and cardiac muscles are also affected and for most boys, respiratory and cardiac failure are major causes of death, often prevalent by the age of 20.

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 possible path forward to approve Kyndrisa in the United States and the review of the marketing authorization application in Europe. 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 Kyndrisa; the content and timing of decisions by the FDA, the EMA and other regulatory authorities concerning Kyndrisa; 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.

Kyndrisa™ is our trademark, and BioMarin® is a registered trademark of BioMarin Pharmaceutical Inc.

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