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For Immediate Release

BioMarin Announces Withdrawal of Market Authorization Application for Kyndrisa™ (drisapersen) in Europe

SAN RAFAEL, Calif., May 31, 2016 – BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) announced today that it has withdrawn its Kyndrisa™ (drisapersen) Marketing Authorization Application (MAA) from the European Medicines Agency (EMA) following discussions at the May 2016 Committee for Medicinal Products for Human Use (CHMP) meeting. Those discussions clearly indicated that the CHMP intended to issue a negative opinion. Kyndrisa is an experimental drug for the treatment of Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping.

Based on discussions at the CHMP meeting and the Food and Drug Administration Complete Response Letter in January, BioMarin intends to discontinue clinical and regulatory development of Kyndrisa as well as the three other first-generation follow-on products, BMN 044, BMN 045 and BMN 053, currently in Phase 2 studies for distinct forms of Duchenne muscular dystrophy. Notwithstanding this outcome for Kyndrisa in Europe, the Company continues to expect to achieve non-GAAP break-even or better in 2017.

BioMarin plans to work with physicians, patient groups, and regulatory authorities to develop a transition plan for those patients currently being treated with Kyndrisa, BMN 044, BMN 045 and BMN 053. The Company will continue to explore the development of next generation oligonucleotides for the treatment of Duchenne muscular dystrophy.

“The withdrawal of the MAA and discontinuation of our current experimental drugs for Duchenne is a difficult but necessary decision at this time,” said Jean-Jacques Bienaimé, BioMarin chairman and chief executive officer. Mr. Bienaimé added, “We want to extend our sincere gratitude to all of the families and caregivers who supported our efforts over the last year to bring Kyndrisa to patients with Duchenne. Our plan now is to invest in research of next generation oligonucleotides with the goal of making a safe and effective treatment available for boys with this devastating disorder.”

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

Duchenne 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 people with serious and life-threatening rare disorders. 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. 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 future development of oligonucleotides for the treatment of DMD. 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 research and preclinical studies related to such product candidates; the content and timing of decisions by regulatory authorities related to such product candidate; 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 2015 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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