MHRA Completes Review of CTA and Issues Notice of Acceptance for BMN 673 for Genetically-Defined Cancers

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NOVATO, Calif., Dec. 2, 2010 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (Nasdaq: BMRN) today announced that the Medicines and Healthcare Products Regulatory Agency (MHRA) in the UK has completed review of the Clinical Trial Application (CTA) and has issued a notice of acceptance for BMN 673 for genetically-defined cancers. The company expects to initiate a Phase 1/2 trial by the first quarter of 2011.

"BMN 673 has been proven to be highly active in mouse xenograft models of human cancer and appears to have superior potency, selectivity, and bioavailability as compared to other products in development," said Hank Fuchs, Chief Medical Officer of BioMarin. "We are eager to get this trial underway and to identify specific tumor types that are more susceptible to treatment with BMN 673. We have built a deep R&D pipeline and remain focused on successfully and expeditiously executing on the programs."

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises four approved products and multiple clinical and pre-clinical product candidates. Approved products include Naglazyme® (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for mucopolysaccharidosis I (MPS I), a product which BioMarin developed through

a 50/50 joint venture with Genzyme Corporation; Kuvan® (sapropterin dihydrochloride) Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; and Firdapse™ (amifampridine phosphate), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Other product candidates include GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in clinical development for the treatment of MPS IVA, and PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase II clinical development for the treatment of PKU. 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: the expectations of the development of BMN 673, including the timing of the clinical trials of the candidate, and the possible efficacy of such candidate. 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: the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities, results and timing of current and planned clinical and preclinical studies related to such product; our ability to successfully manufacture the product; 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 2009 Annual Report on Form 10-K, and the factors contained in BioMarin's reports on Form 10-Q. 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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