# FDA Receives Paragraph IV Notice Letter for KUVAN(R) (sapropterin dihydrochloride) Tablets

SAN RAFAEL, Calif., Sept. 24, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that a Paragraph IV Certification Notice Letter was submitted to the US Food and Drug Administration (FDA) in connection with an Abbreviated New Drug Application (ANDA) to the FDA for approval to market a generic version of KUVAN®(sapropterin dihydrochloride) Tablets, although it has not yet received notice of the certification.

BioMarin has 8 patents listed in the FDA Orange Book with expiration dates between 2024 and 2026. BioMarin will evaluate the Paragraph IV certification when it receives the Notice Letter and intends to vigorously enforce its intellectual property rights. By statute, if BioMarin initiates a patent infringement lawsuit against the party submitting the letter within 45 days of receiving the notice letter, then the FDA would be automatically precluded from approving the ANDA for 30 months, or until a district court decision finding the patents invalid or not infringed, whichever occurs earlier. Once the lawsuit is filed, the 30 month stay period will begin as of the date BioMarin was notified of the filing.

"We believe that our patents related to KUVAN are valid and enforceable and intend to assert our rights vigorously." said Jean-Jacques Bienaimé, BioMarin's CEO. "We have thoroughly prepared for this possibility and intend to file suit within the necessary time frame."

**About BioMarin**

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises five 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) Powder for Oral Solution and Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; Firdapse® (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS); and VIMIZIM® (elosulfase alfa) for the treatment of Morquio A (MPS IVA). Product candidates include: BMN 165 (PEGylated recombinant phenylalanine ammonia lyase), also referred to as PEG PAL, which is currently in Phase 3 clinical development for the treatment of PKU; talazoparib (BMN 673), a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer; BMN 701, a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin like growth factor 2, which is currently in Phase 3 clinical development for the treatment of Pompe disease; BMN 111, a modified C-natriuretic peptide, which is currently in Phase 2 clinical development for the treatment of achondroplasia; and BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1), which is currently in Phase 1 for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease.

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, implied statements regarding the enforcement of our intellectual property rights. These forward-looking statements are subject to certain risks and uncertainties, such as those described in our periodic and other reports filed with the Securities and Exchange Commission, that could cause actual results to differ materially from anticipated results. These risks and uncertainties include, among others, our ability to successfully enforce our intellectual property rights, 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 2013 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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