BioMarin Reviews Status of Exon 51 Composition of Matter and Method of Use Patent Interference Cases against Sarepta Therapeutics

BioMarin to Seek Review of Ruling in Composition of Matter Patent Interference (No. 106,008) Patent Trial and Appeal Board Ruled in BioMarin's Favor on Method of Use (MOU) Patent Interference (No. 106,013)

SAN RAFAEL, Calif., Sept. 21, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that it intends to seek a review of the Patent Trial and Appeal Board (PTAB) of the United States ruling in Interference No. 106,008, related to composition of matter (COM) claims related to exon 51 skipping antisense oligonucleotides. BioMarin is completing its review of the decision and the specific means it may use to seek a further review.

BioMarin also notes that on September 29, 2015, the PTAB ruled in BioMarin's favor in Interference No. 106,013 directed to the method of use (MOU) of exon 51 skipping antisense oligonucleotides to treat Duchenne muscular dystrophy. BioMarin believes that the ruling on the exon 51 COM is specific to those claims and is not relevant to the appeal of the PTAB decision in the MOU interference. BioMarin anticipates a final ruling from the Federal Circuit Court of Appeals on the PTAB decision related to the MOU patent in late 2017 or early 2018. If BioMarin is successful in this appeal, it believes that EXONDYS 51™ (eteplirsen) would infringe the MOU patents.

Companion European Patent
In Europe, BioMarin's method patent EP 1 619 249 B1 was previously upheld in an amended form after an opposition procedure initiated by AVI Biopharma (now Sarepta) before the European Patent Office Opposition Division. The upheld claims include the use of 14- to 40-mer antisense oligonucleotides directed to exon 51 in the DMD gene as a potential therapy to treat DMD. Although an appeal procedure is pending, BioMarin currently has an issued and enforceable patent, which encompasses antisense oligonucleotide product/product candidates directed to exons 51 and 46 in Europe.

About Interference Proceedings in the U.S.
A patent interference is a proceeding conducted by the Patent Office in instances where two parties claim patent rights to the same subject matter. The U.S. patent system awards patents to the first party to invent a particular technology. In an interference, the Patent Office determines which party invented the technology first, and awards the patent to that party, although a party may request that the PTAB consider certain additional matters related to the validity of a patent.

About the Allowed U.S. Patent Application claims

U.S. Patent Application No. 14/198,992 (methods of use) belongs to a portfolio of patents entitled "Modulation of exon recognition in pre-mRNA by interfering with the secondary RNA structure" that was exclusively licensed from the Leiden University Medical Center (LUMC, Leiden, The Netherlands), also referred to as Academisch Ziekenhuis Leiden . The inventor of this application and current BioMarin employee, Dr. Judith van Deutekom, VP, Discovery in Research & Development, was former head of the DMD Genetic Therapy group at LUMC and is one of the pioneers in the development of genetic therapies for muscular dystrophies.

U.S. Patent Application No. 13/550,210 (composition of matter) belongs to the same portfolio of patents that includes the MOU ‘992 application. Thus, the U.S. Patent Application No. 14/198,992 (methods of use) and U.S. Patent Application No. 13/550,210 (composition of matter) have the same priority date.

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.

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 ongoing patent disputes in both Europe and the United States and the scope of the patents and patent applications. 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: future outcomes of patent proceedings, and specifically any challenges to the validity of the patent matters, and any appeals thereto; 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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