BioMarin Receives Favorable Ruling in the Use of Exon 51 Antisense Oligonucleotides Patent Interference

Priority of U.S. Patent Claims Confirmed by U.S. Patent Trial and Appeal Board (PTAB)

SAN RAFAEL, Calif., Sept. 29, 2015 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that the Patent Trial and Appeal Board (PTAB) issued a decision in favor of BioMarin's claims to the use of exon 51 antisense oligonucleotides to treat Duchenne muscular dystrophy. The BioMarin patent application involved is U.S. Patent Application No. 14/198,992 (allowed methods of use claims), which is licensed to BioMarin from the Academisch Ziekenhuis Leiden (AZL), The Netherlands. The decision by the PTAB is based on motions filed in the patent interference proceeding between BioMarin (due to the acquisition of Prosensa Therapeutics N.V. (Prosensa)) and Sarepta (formerly AVI) of Cambridge, Mass as licensee under patents filed by the University of Western Australia (UWA). The allowed claims cover the use of the antisense oligonucleotide drisapersen, as well as other antisense oligonucleotides that induce skipping exon 51 for the treatment of Duchenne muscular dystrophy (DMD). BioMarin also protects drisapersen under U.S. Patents 7,534,879 and 8,759,507, two patents which were not involved in the interference.

The other interference between AZL and UAW related to composition of matter claims for antisense oligonucleotides for Exon 51 skipping, Interference 106,008, remains pending.

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. In the order entered today, the PTAB declared that BioMarin's licensor, AZL, was the first to invent the claimed inventions and therefore entered judgement against the UWA patent application. Sarepta may appeal the PTAB decision to the U.S. Court of Appeals for the Federal Circuit. If they timely file an appeal, it is unlikely that the Patent Office will issue the allowed claims of the AZL patent until the appeal is completed.

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 secondary 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.

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 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 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.

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