BioMarin Completes Enrollment for Phase 3 Trial for GALNS for the Treatment of MPS IVA

Largest Phase 3 ERT Study to Date in a Lysosomal Storage Disease Results Expected in the Fourth Quarter of 2012

NOVATO, Calif. , March 8, 2012 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that enrollment is complete for the pivotal Phase 3 trial for N-acetylgalactosamine 6-sulfatase (GALNS or BMN-110), intended for the treatment of the lysosomal storage disorder Mucopolysaccharidosis Type IVA (MPS IVA), also called Morquio A Syndrome. The company expects to report results in the fourth quarter of 2012.

"It is a major milestone in the GALNS program to have 176 patients randomized and dosed at 31 sites in 17 countries worldwide. We exceeded the planned sample size of 162 patients because physician and patient demand for participation in the trial was extraordinarily high. We were able to accommodate this excess without any delay in our plans to have data available in the fourth quarter this year," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "We would like to thank the investigators, patients and their families, and hardworking employees of BioMarin who have contributed efforts to the largest study in the history of the company. The Phase 3 trial for GALNS is currently the highest development priority at BioMarin, and we have taken every possible measure to design and conduct this study under the highest standards to maximize our chance of success. Along with our supplemental studies for patients under five years of age and non-ambulatory patients, we believe the results from the Phase 3 pivotal study will be well-positioned to support regulatory filings in the first quarter of 2013."

The Phase 3 trial is a randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of GALNS in patients with MPS IVA. The study is exploring doses of two mg/kg/week and two mg/kg/every other week for a treatment period of 24 weeks. The primary endpoint is the six-minute walk test, and the secondary endpoints are the three-minute stair climb test and urine keratan sulfate concentration.

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), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS). Product candidates include GALNS (N-acetylgalactosamine 6-sulfatase), which is currently in Phase III clinical development for the treatment of MPS IVA, amifampridine phosphate (3,4-diaminopyridine phosphate), which is currently in Phase III clinical development for the treatment of LEMS in the U.S., PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase II clinical development for the treatment of PKU, BMN-701, a novel fusion protein of insulin-like growth factor 2 and acid alpha glucosidase (IGF2-GAA), which is currently in Phase I/II clinical development for the treatment of Pompe disease, BMN-673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase I/II clinical development for the treatment of genetically-defined cancers, and BMN-111, a modified C-nutriuretic peptide, which is currently in Phase I clinical development for the treatment of achondroplasia. For additional information, please visit www.BMRN.com. Information on BioMarin's website is not incorporated by reference into this press release.

The BioMarin Pharmaceutical Inc. logo is available at http://www.globenewswire.com/newsroom/prs/?pkgid=11419

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 related to the continued clinical development of its product candidate GALNS; and actions by regulatory authorities. 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 current and planned preclinical studies and clinical trials of GALNS; our ability to successfully manufacture our products and product candidates; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning GALNS 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 2010 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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