BioMarin Initiates Phase 3 Trial for BMN 673 for the Treatment of Metastatic gBRCA Breast Cancer

SAN RAFAEL, Calif., Oct. 31, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that it has dosed the first patient in its Phase 3 program to evaluate BMN 673, its poly ADP-ribose polymerase (PARP) inhibitor, in the treatment of metastatic germline BRCA mutated breast cancer.

"It has been very exciting to work with this novel molecule in the preclinical laboratory where we saw it to be best in class. It is equally exciting to now be involved in the clinical translation of this drug in this genetically defined population of breast cancer patients who may benefit from a treatment option specifically designed for them," said Dennis Slamon, Ph.D., M.D., Chief, Division Hematology/Oncology and Director of Clinical/Translational Research, Jonsson Comprehensive Cancer Center, David Geffen School of Medicine at the University of California, Los Angeles. "Oncology therapy is moving towards a personalized and targeted model with the goal of establishing more effective treatments based on an individual's genetic profile, and BMN 673 could be an important step toward tailoring cancer treatments by tumor type."

"Enrolling the first patient is an important milestone in the clinical trial process. We are excited to be part of a trial that not only offers the potential to eliminate the use of chemotherapy, but also the possibility of improving the length and quality of life for patients already diagnosed with hereditary breast cancer," said Fran Visco, President of the National Breast Cancer Coalition.

The Phase 3 study is an open-label, randomized, parallel, two-arm, multi-center study of BMN 673 *versus* physician's choice in approximately 430 germline BRCA mutation patients with locally advanced and/or metastatic breast cancer, who have received no more than two prior chemotherapy regimens for metastatic disease. The primary objective of the study is to measure progression free survival (PFS). Secondary objectives include evaluating the objective response rate (ORR) and the overall survival (OS).

"We are eager to fully enroll this important trial for breast cancer patients with hereditary breast cancer to better understand the role of BMN 673 in this defined population," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "We are looking forward to gaining a better understanding of the safety and efficacy of our compound."

About Hereditary Breast Cancer with BRCA Mutation

BRCA1 and BRCA2 are human genes that belong to a class of genes known as tumor suppressors. Mutation of these genes has been linked to hereditary breast and ovarian cancer. A woman's risk of developing breast and/or ovarian cancer is greatly increased if she inherits a deleterious (harmful) BRCA1 or BRCA2 mutation. Men with these mutations also have an increased risk of breast cancer. Both men and women who have harmful BRCA1 or BRCA2 mutations may also be at increased risk of other cancers.

Source: National Cancer Institute at the National Institutes of Health

http://www.cancer.gov/cancertopics/factsheet/Risk/BRCA

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 Vimizim (N-acetylgalactosamine 6-sulfatase), formally referred to as GALNS, which successfully completed Phase 3 clinical development for the treatment of MPS IVA, PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase 3 clinical development for the treatment of PKU, 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) wth a peptide derived from insulin like growth factor 2, which is currently in Phase 1/2 clinical development for the treatment of Pompe disease, BMN 111, a modified C-natriuretic peptide, which is currently in Phase 1 clinical development for the treatment of achondroplasia and BMN 190, a

recombinant human tripeptidyl peptidase-1 (rhTPP1) 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, statements about: the enrollment of a Phase III trial for BMN 673 in metastatic gBRCA breast cancer, the expectations of the development of BMN 673, including the possible safety and 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 successful enrollment of the Phase 3 trial; results and timing of current and planned preclinical studies and clinical trials of BMN 673; our ability to successfully manufacture BMN 673; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning BMN 673; 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 2012 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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