BioMarin Provides BMN 673 Program Update

RECIST Response Rate Increases to 50% in gBRCA Breast Cancer; Study Ongoing
Phase 3 Trial to Start in 3Q 2013
National Breast Cancer Coalition Announces Intention to Assist With Trial Recruitment

SAN RAFAEL, Calif., July 25, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today provided an update on its ongoing Phase 1/2 study for its poly ADP-ribose polymerase (PARP) inhibitor BMN 673 for the treatment of solid tumors. As of July 24, the RECIST response rate from the ongoing trial is nine out of 18 breast cancer patients, or 50 percent, including one confirmed complete response. This response rate includes three additional confirmed responses since the last update at the ASCO Annual Meeting, and two new patients are yet to be confirmed. Four patients are ongoing with stable disease with potential for additional responses. The study is still ongoing, and the company will provide additional updates later this year, including data in ovarian cancer, Ewing's sarcoma and small cell lung cancer.

Since the ASCO Annual Meeting, the company has met with the FDA and MAA to review its planned clinical program in deleterious gBRCA mutation metastatic breast cancer. At those meetings, health authorities confirmed that a primary endpoint of Progression Free Survival (PFS) could serve as the basis for a potential approval. BioMarin now expects to initiate a Phase 3 trial for BMN 673 in deleterious gBRCA mutation metastatic breast cancer in late third quarter of 2013, earlier than previously announced at the ASCO Annual Meeting in early June.

National Breast Cancer Coalition (NBCC) has announced its intention to assist BioMarin's development of the PARP inhibitor for the treatment of hereditary breast cancer with BRCA mutations. NBCC intends to participate with BioMarin on enrollment initiatives in the United States and around the world. In addition, NBCC will be a resource on study design, implementation and execution.

"NBCC is an organization committed to ending breast cancer in large part by participating in and contributing to the scientific process. The application of drug development science to well-defined problems is an important step in our mission. Therefore, we are pleased to collaborate with BioMarin to develop BMN 673 as a potential first and/or best in class PARP inhibitor for patients with hereditary forms of cancer, namely BRCA mutations. This project offers the hope of improving the length and/or quality of life for patients," said Fran Visco, President of the National Breast Cancer Coalition.

"We look forward to conducting a world-class study, in collaboration with the finest clinical investigators in the world to evaluate the safety and efficacy of BMN 673 in the metastatic setting. Our advocates are poised to change the world through our actions. Ultimately, we hope to prevent women from getting breast cancer and prevent it from spreading outside the breast," Visco added.

"BioMarin is an organization that wants to make big differences in the lives of patients. The data emerging from the ongoing BMN 673 study is progressively more encouraging. We are committed to the successful execution of the Phase 3 program in hopes of offering patients a safe and easily administered pill, which is an effective treatment option," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "We are honored to include a seat at the table for our colleagues from NBCC. The purpose of our efforts is best served hearing their voice from the beginning and taking action together."

About the National Breast Cancer Coalition (NBCC)

The National Breast Cancer Coalition (NBCC) is dedicated to knowing how to end breast cancer by January 1, 2020 through the power of grassroots action and advocacy. NBCC increases funding for innovative breast cancer research; monitors how those funds are spent; expands access to quality health care for all; and ensures that trained advocates influence all decision making in breast cancer. Join NBCC, learn more and take action visit BreastCancerDeadline2020.org.

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 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 BMN 673 Phase 1 Trial Data

BioMarin is conducting an ongoing Phase 1 study for its poly ADP-ribose polymerase (PARP) inhibitor BMN 673 for the treatment of solid tumors. BMN 673 has exhibited substantial single-agent anti-tumor activity in deleterious germline BRCA breast cancers in the trial. The preliminary data were presented during a poster presentation at the 2013 American Society of Clinical Oncology Annual Meeting on June 3, 2013.

The Phase 1 trial is an open-label study of once-daily, orally-administered BMN 673 in approximately 70 patients ages 18 and older with advanced or recurrent solid tumors. The primary objective of the study is to establish the maximum tolerated dose (MTD) of daily oral BMN 673. The secondary objectives are to assess the safety, tolerability, preliminary efficacy and pharmacodynamic activity of BMN 673, and to determine the pharmacokinetic profile. The study design was a standard 3 + 3 dose escalation followed by expansion at MTD in cohorts with selected tumor types to further characterize safety and anti-tumor activity.

BMN 673 was generally well-tolerated. The dose-limiting toxicity was Grade 4 thrombocytopenia. Myelosuppression, most of which was moderate in severity, occurred in 10-20% of patients with chronic dosing. Fatigue, nausea and alopecia were observed in 20-30% of patients. Signs of activity were seen at doses as low as 100 µg/day, and the maximum tolerated dose was 1000 mg/day (1.0 mg/day), which is the expected dose for further development. BMN 673 also has good bioavailability and a long half-life which supports once-daily dosing.

To access the poster presented at ASCO

http://www.bmrn.com/pipeline/clinical-trials/asco.php

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 III clinical development for the treatment of MPS IVA, PEG-PAL (PEGylated recombinant phenylalanine ammonia lyase), which is currently in Phase III 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-natriuretic 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.

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 of the development of BMN 673, including the timing of the clinical trials of the candidate, and the possible safety and efficacy of such candidate, and the participation of NBCC in the trial execution. 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 content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities, results and timing of current and planned clinical and preclinical studies related to such product; our ability to successfully manufacture the product; NBCC actual level of involvement with the trials; 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.

BioMarin Pharmaceutical Inc.

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