BioMarin Provides Preliminary Data on Ongoing Phase 1/2 Trial for BMN 673 for the Treatment of Solid Tumors at 2013 American Society of Clinical Oncology Annual Meeting

Phase 3 Trial in Metastatic gBRCA Breast Cancer Planned to Start in Q4 2013

SAN RAFAEL, Calif., June 3, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced an update on the ongoing Phase 1/2 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 ovarian and breast cancers in the trial. The data were presented during a poster presentation at the 2013 American Society of Clinical Oncology Annual Meeting.

In the 28 gBRCA ovarian cancer patients, the RECIST response rate was 44% or 11 of 25 evaluable patients, the CA-125 response rate was 70% or 19 of 27 evaluable patients and the clinical benefit response rate was 82% or 23 of 28 patients.

In the 18 gBRCA breast cancer patients, the RECIST response rate was 39% or 7 of 18 patients and the clinical benefit rate was 67% or 12 of 18 patients. Of the 18 gBRCA breast cancer patients, there were six partial responses (three yet to be confirmed) and one complete response. Four ongoing patients have had stable disease for less than 12 weeks. Treatment is ongoing in 12 of the 18 breast cancer patients in the study.

"Patients with germline BRCA-associated tumors have no targeted treatment options. There is a need for therapies that target specific molecular defects in tumors, and PARP inhibitors offer that potential in BRCA-related cancers. We have seen excellent anti-tumor activity in some of our patients treated with BMN 673. Continued study of this molecule will be meaningful for advancing the care for patients suffering from these cancers," said Professor Johann de Bono, Professor of Experimental Cancer Medicine at The Institute of Cancer Research, London, and Honorary Consultant in Medical Oncology at The Royal Marsden NHS Foundation Trust.

BMN 673 was generally well-tolerated. The dose-limiting toxicity was Grade 3 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 as low as 100 µg/day, and the maximum tolerated dose was 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.

"We are encouraged by this early stage data on BMN 673, including the safety profile, and substantial anti-tumor activity. We look forward to initiating a Phase 3 trial in metastatic gBRCA breast cancer patients to pursue a safe and effective therapy in a once-daily, oral dose that meets an unmet medical need in this aggressive form of cancer," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin.

More complete data in breast and ovarian cancers, as well as Ewings sarcoma and small cell lung cancer (SCLC) will be presented at a future medical meeting.

Based on this preliminary data, BioMarin expects to start a Phase 3 trial in gBRCA breast cancer in the fourth quarter of 2013.

Study Objectives and Design

The Phase 1/2 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.

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. 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; 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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