BioMarin Provides Updated Phase 1/2 Data on BMN 673 in Breast Cancer at the European Cancer Congress 2013

Confirmed RECIST Response Rate of 50% in gBRCA Breast Cancer Patients Treated With 1mg/day Phase 3 Dose
Overall Benefit Response Rate With 1mg/day Phase 3 Dose of 86%

SAN RAFAEL, Calif., Sept. 29, 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. Dr. Ramesh Ramanathan, lead investigator of the study (Medical Director, Virginia G. Piper Cancer Center - Clinical Trials, Scottsdale Health Care, Scottsdale, AZ and Clinical Professor of Medicine, TGEN & College of Medicine, University of Arizona, Phoenix, AZ), presented the data at the Late Breaking Oral Session of the the 17th ECCO — 38th ESMO — 32nd ESTRO European Cancer Congress in Amsterdam, The Netherlands.

Data Presented Today

In the most currently available data presented today from the ongoing Phase 1/2 study, among 14 gBRCA breast cancer patients treated at the dose of 1mg/day selected for the Phase 3 study, the confirmed RECIST response rate was 50% (7 confirmed objective responses: 1 complete and 6 partial). In addition, there were 5 patients with stable disease lasting at least 24 weeks for an overall clinical benefit response rate at this dose of 86% (12/14). Responses presented in the oral presentation are all confirmed responses.

The median progression-free survival (PFS) has not yet been reached for the gBRCA breast cancer patients. It is anticipated that the median PFS will exceed 6 months in this heavily pre-treated patient population.

In the complete cohort of 18 gBRCA breast cancer patients, which included 6 patients from the dose escalation cohort at doses ranging from 900 µg to 1100 µg and 12 patients from the dose expansion cohort at a dose of 1.0 mg, the RECIST response rate was 44% or 8 of 18 patients with 1 complete and 7 partial responses. The clinical benefit rate was 72% or 13 of 18 patients with 5 patients having stable disease in excess of 24 weeks. Treatment is ongoing in 9 of the 18 breast cancer patients in the study.

At all doses (n=18) there has been a best response of partial response or better in 12 patients, and four patients progressed prior to confirmation. Of the 14 patients treated at 1mg, there has been a best response of partial response or better in 8 patients, and one patient progressed prior to confirmation.

BMN 673 was generally well-tolerated. The dose-limiting toxicity was thrombocytopenia. Myelosuppression was generally mild-to-moderate in severity. Greater than grade 1 anemia, thrombocytopenia and neutropenia occurred in 25%, 20% and 12.5% of patients, respectively, with chronic dosing. Fatigue, nausea and alopecia were observed in 26-29% 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 the data presented at the European Cancer Congress, including the safety profile, and significant anti-tumor activity," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "With the imminent initiation of the Phase 3 program in germline BRCA mutation metastatic breast cancer, we are closer to understanding the significant role BMN 673 may play for patients suffering metastatic breast cancer."

Abstract with Preliminary Data Published

The abstract 29LBA, *PARP inhibition with BMN 673 in ovarian and breast cancer patients with deleterious mutations of BRCA1 and BRCA2,* has been posted to the website (www.ecco-org.eu) of the 17th ECCO — 38th ESMO — 32nd ESTRO European Cancer Congress in Amsterdam, The Netherlands. The published abstract 29LBA included preliminary data as of the July 29, 2013 submission date. This submission included responses that were unconfirmed at that time.

Phase 3 Trial Design

The Phase 3 trial, which will began enrolling patients imminently, is an open-label, 2:1 randomized, parallel, two-arm study of BMN 673 as compared to physicians' choice (capecitabine, eribulin, gemcitabine or vinorelbine) in germline BRCA mutation subjects with locally advanced and/or metastatic breast cancer who

have received no more than two prior chemotherapy regimens for metastatic disease. The study is enrolling approximately 429 subjects and is being conducted at approximately 100 sites in twelve countries. The primary objective of the study is to compare progression-free survival (PFS) of subjects treated with BMN 673 as a monotherapy relative to those treated with protocol-specified physicians' choice. The secondary objectives are to evaluate objective response rate (ORR), overall survival (OS), safety and the pharmacokinetics of BMN 673.

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

BioMarin is conducting an ongoing Phase 1 study, PRP-001, for its poly ADP-ribose polymerase (PARP) inhibitor BMN 673 for the treatment of solid tumors. The PRP-001 Phase 1 trial is an open-label study of once-daily, orally-administered BMN 673 in approximately 80 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 is 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.

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 expected data to be presented at the congress, the expectations of the development of BMN 673, including the timing of the clinical trials of the candidate, the possible safety and efficacy of such candidate, and the observed and expected response rate of the 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: confirmation of observed responses, 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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