BioMarin Announces BMN-673 Data in Breast and Ovarian Cancers to be Released at ASCO

Phase 3 Trial in gBRCA Breast Cancer Planned for 4Q 2013

SAN RAFAEL, Calif., May 16, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that data from its ongoing Phase 1/2 trial for BMN-673 for genetically-defined cancers will be released at the American Society of Clinical Oncology (ASCO) meeting in Chicago on June 3, 2013. At ASCO, the company will present data from 18 gBRCA breast cancer patients, including six patients from the dose escalation cohort at doses ranging from 25 µg to 1100 µg and twelve patients from the dose expansion cohort at a dose of 1.0 mg. Data will also be presented from 28 gBRCA ovarian cancer patients, including 17 patients from the dose escalation cohort and 11 patients from the dose expansion cohort.

ASCO Data Presentation Details

Date: June 3, 2013

Time: 10:00 a.m. - 1:45 p.m.

Session: Developmental Therapeutics - Clinical Pharmacology and Experimental Therapeutics

Abstract #2580 (Temp. Abst. ID: 111725): First-in-human trial of novel oral PARP inhibitor BMN 673 in patients with solid tumors.

Data to be presented at ASCO will be early dose expansion data from the ongoing study, with more mature data in breast and ovarian cancers, as well as initial data in Ewings sarcoma and small cell lung cancer (SCLC) to be presented at the European Society for Medical Oncology in Amsterdam in September 2013.

"We look forward to providing a more complete picture of our ongoing BMN-673 Phase 1/2 program at ASCO with additional data from patients in the dose expansion cohort," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "We believe that the selectivity and extreme potency of BMN-673 provide a good basis for it to potentially emerge as the best-in-class compound, and we are committed to moving forward with a Phase 3 program in gBRCA breast cancer by the end of the year."

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 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-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 regarding the exact data to be presented at the upcoming ASCO meeting. 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 timing and outcome of the clinical trials of BMN-673 and the data available at the time of publication; and those risks that are discussed in BioMarin's filings with the Securities

and Exchange Commission, including, without limitation, BioMarin's 2012 Annual Report on Form 10-K, and our periodic reports on Form 10-Q and Form 8-K. 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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