BioMarin Announces Oral Presentation of BMN 673 Most Recent Data on Breast and Ovarian Cancers at the European Cancer Congress 2013

Late Breaking Abstract on PARP Inhibition

SAN RAFAEL, Calif., Aug. 16, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that its abstract, *PARP inhibition with BMN 673 in ovarian and breast cancer patients with deleterious mutations of BRCA1 and BRCA2* has been selected as a late breaking abstract by the 17th ECCO — 38th ESMO — 32nd ESTRO European Cancer Congress in Amsterdam, The Netherlands and will be presented in an oral presentation on September 29, 2013.

The company will present the latest data from its ongoing Phase 1/2 trial for genetically-defined cancers, including data from 18 breast cancer patients with deleterious germline BRCA mutations, including six patients from the dose escalation cohort at doses ranging from 900 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 ovarian cancer patients with deleterious germline BRCA mutations, including 17 patients from the dose escalation cohort (range 100 µg to 1100 µg) and 11 patients from the dose expansion cohort.

European Cancer Congress Data Presentation Details

Date:	September 29, 2013
Time:	10:45 a.m.
Late Breaking Abstract Title:	*PARP inhibition with BMN 673 in ovarian and breast cancer patients with deleterious mutations of BRCA1 and BRCA2*
Session:	Proffered paper session on "Breast Cancer — Early Disease" (Oral Presentation)
Abstract #	29LBA
	The European CanCer Organisation (ECCO) intends to publish abstracts online, for general viewing on its website (www.ecco-org.eu).

Data to be presented at the European Cancer Congress will be early dose expansion data from the ongoing study, with more mature data in breast and ovarian cancers.

"We are delighted that the European Cancer Congress has included an update to our BMN 673 data on our Phase 1/2 program as a late-breaking abstract at this important medical meeting," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "We are on track to initiate a Phase 3 program in germline BRCA mutation metastatic breast cancer by the end of September and continue to see potential for BMN 673 to be the best-in-class PARP inhibitor."

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 preliminary data were presented during a poster presentation at the 2013 American Society of Clinical Oncology Annual Meeting on June 3, 2013.

The PRP-001 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 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, 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.

CONTACT: Investors and Media:

Debra Charlesworth

BioMarin Pharmaceutical Inc.

(415) 455-7451

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https://investors.biomarin.com/2013-08-16-BioMarin-Announces-Oral-Presentation-of-BMN-673-Most-Recent-Data-on-Breast-and-Ovarian-Cancers-at-the-European-Cancer-Congress-2013