

Five Data Presentations on BioMarin's BMN 673 PARP Inhibitor at the 2013 AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics

BMN 673 Potentially 100-Fold More Potent Than Other PARP Inhibitors

SAN RAFAEL, Calif., Oct. 20, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) today announced that five poster presentations on BMN 673 will be featured at the 2013 AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics in Boston from October 19-23, 2013. The five pre-clinical and clinical data abstracts include:

- *Stereospecific Trapping of PARP-DNA Complexes by BMN 673 and Comparison with Olaparib and Rucaparib* (Abstract A257) to be presented on Sunday, October 20, 2013 from 12:30 — 3:00 PM.
- *Inhibition of PBMC PARP activity with the novel PARP 1/2 inhibitor BMN 673 in patients with advanced solid tumors*(Abstract A220) to be presented on Sunday, October 20, 2013 from 12:30 — 3:00 PM.
- *Preclinical evaluation of BMN 673 in combination with temozolomide (TMZ) in various tumor types including small cell lung cancer (SCLC) cells* (Abstract B93) to be presented on Monday, October 21, 2013 from 12:30 PM - 3:00 PM
- *Pediatric Preclinical Testing Program (PPTP) evaluation of BMN 673, an inhibitor of Poly-ADP Ribose Polymerase (PARP), alone and with Temozolomide (TMZ)* (Abstract C206) to be presented Tuesday, October 22, 2013 from 12:30 PM - 3:00 PM
- *Update on first-in-human trial of novel oral PARP inhibitor BMN 673 in patients with solid tumors* (Abstract C295) to be presented on Tuesday, October 22 from 12:30-3:00.

Junko Murai and Yves Pommier from the National Cancer Institute, will present *Stereospecific trapping of PARP-DNA complexes by BMN 673 and comparison with olaparib and rucaparib*. The research as reported on the poster concluded that BMN 673 was approximately 100-fold more potent than olaparib and rucaparib at trapping PARP, making it the most potent clinical PARP inhibitor to date with the highest efficiency at trapping PARP-DNA complexes.

PARP-DNA trapping helps explain why PARP inhibitors show different potency in killing tumor cells, even when they are similar in inhibiting enzymatic activity in PARP. While reducing the level of PARP enzymatic activity seems to be important, trapping PARP onto DNA is lethal to the cancer cell if not repaired. Tumor cells defective in DNA repair function are more sensitive to PARP inhibitors than normal cells with full DNA repair capability. The research concluded that PARP inhibitors should be evaluated on both catalytic PARP inhibition and PARP-DNA trapping to fully understand the impact.

"We believe PARP trapping seen with BMN 673 is differentiating because BMN 673 appears to be significantly more lethal to cancer cells than olaparib and rucaparib, and PARP trapping may explain why BMN 673 shows far greater potency," said Hank Fuchs, M.D., Chief Medical Officer at BioMarin. "We're looking forward to further studies in gBRCA metastatic breast cancer patients to confirm and extend the early clinical results of the compound."

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, BMN-111, a modified C-natriuretic peptide, which is currently in Phase I clinical development for the treatment of achondroplasia and BMN 190, a recombinant human tripeptidyl peptidase-1 (rhTPP1) for the treatment of late-infantile neuronal ceroid lipofuscinosis (CLN2), a form of Batten Disease. 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 data presented at the conference, the expectations of the development of BMN 673, including the possible safety and efficacy of such candidate, and the observed and expected potency and anti-tumor activity 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: 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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