Sarah Cannon Research Institute UK and BioMarin Collaborate on EMBRACA Clinical Study in Hereditary Breast Cancer With BRCA Mutation

Patient Enrollment Expands Internationally With First Patient Outside of U.S.

SAN RAFAEL, Calif., July 23, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) and Sarah Cannon Research Institute UK today announced a collaboration to enroll patients in an ongoing Phase 3 clinical trial of its PARP inhibitor, BMN 673, for the treatment of hereditary breast cancer with a BRCA mutation. This ongoing Phase 3 trial has recently been named EMBRACA. Sarah Cannon Research Institute UK enrolled the first patient outside of the United States, expanding the trial internationally.

"We are excited to collaborate with BioMarin on this landmark trial to increase the treatment opportunities for patients with BRCA related breast cancer," said Dr Alison Jones, Consultant Medical Oncologist and Principal Investigator for the EMBRACA Phase 3 trial at Sarah Cannon Research Institute UK.

"This study is an important milestone for both organizations to foster future collaborations," highlighted Dr Hendrik-Tobias Arkenau, FRCP, PhD, Medical Oncologist and Medical Director at Sarah Cannon Research Institute UK.

"Sarah Cannon Research Institute UK has a long history of pioneering significant advances in medical therapy, and we are thrilled to commence enrollment outside of the United States to evaluate the safety and efficacy of BMN 673 in patients with hereditary breast cancer," said Hank Fuchs, M.D., Chief Medical Officer of BioMarin. "Breast cancer patients with germline BRCA-associated tumors have no targeted treatment options. There is an unmet need for therapies that target specific molecular defects in tumors, and PARP inhibitors offer that potential in BRCA-related breast cancer."

The EMBRACA Phse 3 study began in the United states in the fall of 2013 and has expanded internationally. BioMarin plans to enroll patients from sites around the world and to work with partners outside of the United States. The EMBRACA Phase 3 study is an open-label, randomized, parallel, two-arm, multi-center study of BMN 673 versus physician's choice in approximately 430 germline BRCA mutation patients with locally advanced and/or metastatic breast cancer, who have received no more than two prior chemotherapy regimens for metastatic disease. The primary objective of the study is to measure progression free survival (PFS). Secondary objectives include evaluating the objective response rate (ORR) and the overall survival (OS).

About BMN 673

BMN 673 is an inhibitor of PARP, poly-ADP ribose polymerase, an enzyme involved in the repair of DNA damage that is essential for survival of some types of tumor cells. BMN 673 is the most potent PARP inhibitor reported for killing tumor cells in preclinical and clinical settings, which has been explained by investigators at the National Cancer Institute as due to efficient trapping of PARP-DNA complexes [i], [ii] ,[iii]. BMN 673 has shown single agent activity in breast and ovarian cancer in BRCA carrier patients, and also in small cell lung cancer.

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 also be at increased risk of other cancers.[iv]

About Sarah Cannon Research Institute UK

Sarah Cannon Research Institute UK (SCRI UK) specializes in the development of novel cancer therapies by providing clinical research and personalized medicine options for cancer patients throughout London and the United Kingdom. As the first unit outside the NHS to offer new anti-cancer drugs in clinical trials, SCRI UK provides rapid access to investigational drug therapies open to both private and NHS patients. SCRI UK is the international research programme of Sarah Cannon Research Institute, the global strategic research organization focusing on advancing therapies for patients. As one of the largest clinical research programs worldwide, SCRI conducts community-based clinical trials in oncology and cardiology through affiliations with a network of more than 700 physicians in the United States and United Kingdom. For more information, please visit sarahcannonresearch.co.uk.

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio comprises five 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) Powder for Oral Solution and Tablets, for phenylketonuria (PKU), developed in partnership with Merck Serono, a division of Merck KGaA of Darmstadt, Germany; Firdapse® (amifampridine), which has been approved by the European Commission for the treatment of Lambert Eaton Myasthenic Syndrome (LEMS); and VIMIZIM® (N-acetylgalactosamine 6-sulfatase) for the treatment of Morquio A (MPS IVA). Product candidates include: BMN 165 (PEGylated recombinant phenylalanine ammonia lyase), also referred to as PEG PAL, which is currently in Phase 3 clinical development for the treatment of PKU; BMN 673, a poly ADP-ribose polymerase (PARP) inhibitor, which is currently in Phase 3 clinical development for the treatment of germline BRCA breast cancer; BMN 701, a novel fusion of acid alpha glucosidase (GAA) with a peptide derived from insulin like growth factor 2, which is currently in Phase 3 clinical development for the treatment of Pompe disease; BMN 111, a modified C-natriuretic peptide, which is currently in Phase 1 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 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 2013 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.

[i] Shen Y, Rehman FL, Feng Y, Boshuizen J, Bajrami I, Elliott R, Wang B, Lord CJ, Post LE, Ashworth A., Clin Cancer Res. 2013 Sep 15;19(18):5003-15. doi: 10.1158/1078-0432.CCR-13-1391. Epub 2013 Jul 2

[ii] Wainberg Z, Rafii S, Ramanathan R K, Mina L, Averett Byers L, Chugh R, Goldman J W, Sachdev J, Matei D, Wheler J J Henshaw J W, Zhang C, Gallant G J A, de Bono J S, *Safety and Antitumor Activity of the PARP inhibitor BMN 673 in a Phase 1 Trial Recruiting Metastatic Small-Cell Lung Cancer (SCLC) and Germline BRCA Mutation Carrier Cancer Patients*, Abstract 7522 presented at American Society of Clinical Oncology Annual Meeting, Poster Highlights Session: Lung Cancer - Non-small Cell Local-regional/Small Cell/Other Thoracic Cancers, May 30 - June 3, 2014

[iii] Murai J, Huang SY, Renaud A, Zhang Y, Ji J, Takeda S, Morris J, Teicher B, Doroshow JH, Pommier Y., Mol Cancer Ther. 2014 Feb;13(2):433-43. doi: 10.1158/1535-7163.MCT-13-0803. Epub 2013 Dec 19

[iv] National Cancer Institute at the National Institutes of Health, http://www.cancer.gov/cancertopics/factsheet/Risk/BRCA

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