BioMarin Announces Selection of Factor VIII Gene Therapy Drug Development Candidate BMN 270 for the Treatment of Hemophilia A

SAN RAFAEL, Calif., Jan. 13, 2014 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that it has selected an AAV-factor VIII vector, BMN 270, to develop for the treatment of hemophilia A and has initiated IND-enabling studies. BioMarin expects to initiate clinical studies with BMN 270 in early 2015. The Company's gene therapy program for hemophilia A was originally licensed from University College London (UCL) and St. Jude Children's research Hospital in February 2013 and has since been developed at BioMarin's facilities.

"In our hemophiliac factor VIII deficient mouse models, BMN 270 restored factor VIII plasma concentrations to levels projected to be adequate for normal clotting in humans," said Barrie Carter, Ph.D., Vice President, Vector Biology, of BioMarin. "In the past eleven months since we acquired the AAV-factor VIII technology from UCL and St. Jude, we have evaluated a large number of candidate vectors for treating hemophilia A, and we are pleased to have selected BMN 270 as BioMarin's next IND candidate."

"Gene therapy is emerging as a powerful way to treat genetic disorders and is complementary to our current suite of commercial products and research programs," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "Hemophilia is an attractive target for gene therapy as factor levels in the blood serve as good biomarkers, relatively low factor levels are required for a clinically important benefit and the current standard of care of multiple intravenous infusions per week is quite onerous for patients. We remain committed to maintaining a rich pipeline with the goal of filing an IND every twelve to eighteen months."

Professor Amit Nathwani, Ph.D., at University College London added, "UCL is a world leader in the biomedical sciences, with an unremitting commitment to outstanding research and translation into healthcare benefits for patients. We are pleased to collaborate with BioMarin in this exciting field of gene therapy for hemophilia and keen to see a revolution in treatment for this debilitating condition."

About Hemophilia A

The current market for hemophilia A products is about $6.0 billion worldwide. There are approximately 90,000 patients in territories where BioMarin has commercial operations with an annual incidence of about 400 new patients in the U.S. The standard of care for the 60 percent of hemophilia A patients who are severely affected is a prophylactic regimen of IV infusions three times per week. Even with prophylactic regimens, many patients still experience spontaneous bleeding events that result in progressive and debilitating joint damage due to result of chronically low factor levels.

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 MPS VI, a product wholly developed and commercialized by BioMarin; Aldurazyme® (laronidase) for 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 3 clinical development for the treatment of MPS IVA, PEG PAL (PEGylated recombinant phenylalanine ammonia lyase), 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 1/2 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 BMN 270, including the expected timing of the pre-clinical trials and initiation of clinical trials 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: the results of current and ongoing preclinical trials, particularly the IND-enabling toxicology; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities; our ability to successfully manufacture the product candidtate for the preclinical and clinical trials; 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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