BioMarin Licenses Factor VIII Gene Therapy Program for Hemophilia A From University College London and St. Jude Children's Research Hospital

SAN RAFAEL, Calif., Feb. 21, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today that it has licensed a Factor VIII gene therapy program for hemophilia A from University College London (UCL) and St. Jude Children's Research Hospital. The company expects to select a development candidate this year, initiate and complete IND-enabling toxicology studies next year and initiate proof of concept human studies by the end of 2014. The license and commitment to support the research program was made possible by UCL Business, UCL's wholly-owned technology transfer company, working with Professor Amit Nathwani of the UCL Cancer Institute.

"Gene therapy is emerging as a powerful and viable 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 in severe patients and the current standard of care of intravenous infusions three times a week is quite onerous. We remain committed to maintaining a rich pipeline with the goal of filing an IND every twelve to eighteen months."

Mr. Cengiz Tarhan, Managing Director of UCL Business said, "This is an excellent partnership for UCL Business, which combines the world class translational research strengths of Professor Nathwani and his team with the significant development and commercialization capabilities of BioMarin to progress this ground breaking therapy for hemophilia A."

Professor Stephen Caddick, Vice-Provost (Enterprise) at University College London added, "UCL and BioMarin each bring distinct strengths to the partnership. UCL is a world leader in the biomedical sciences, with an unremitting commitment to outstanding research and translation into healthcare benefits for patients. We welcome this partnership which will continue to build on the excellence of our research to fully explore the potential of gene therapy as a life-saving treatment for people with hemophilia."

Andrew Davidoff, M.D., Chair, Surgery, St. Jude Children's Research Hospital, added, "We are pleased that our research with UCL on gene therapy for hemophilia has led to the development of a potential therapeutic tool for treating this devastating disease. This licensing agreement underscores St. Jude's commitment to rapidly translating our research into effective clinical interventions."

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 and 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 severe is a prophylactic regimen of IV infusions three times per week. Even with the likely prospect of less frequently dosed products coming to the market, feedback from thought leaders indicates that significant unmet need will remain as factor replacement therapy will inevitably leave patients vulnerable to bleeding events. Many patients on factor replacement therapy still have bleeding events and experience debilitating damage to joints as a 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 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 BMN-110 (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.

The BioMarin Pharmaceutical Inc. logo is available at http://www.globenewswire.com/newsroom/prs/?pkgid=11419

About UCLB

UCLB is a leading technology transfer company that supports and commercialises research and innovations arising from UCL, one of the UK's top research-led universities.

UCLB has a successful track record and a strong reputation for identifying and protecting promising new technologies and innovations from UCL academics. It invests directly in development projects to maximise the potential of the research and manages the commercialisation process of technologies from the laboratory to market.

UCLB supports UCL's Grand Challenges of increasing UCL's positive impact on and contribution to Global Health, Sustainable Cities, Intercultural Interaction and Human Wellbeing.

For further information, please visit www.uclb.com

St. Jude Children's Research Hospital

St. Jude Children's Research Hospital is internationally recognized for its pioneering research and treatment of children with cancer and other life-threatening diseases. The hospital's research has helped push overall survival rates for childhood cancer from less than 20 percent when the institution opened to almost 80 percent today. It is the first and only National Cancer Institute-designated Comprehensive Cancer Center devoted solely to children, and no family ever pays St. Jude for anything. For more information, visit www.stjude.org. Follow us on Twitter @StJudeResearch.

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: expectations related to the development of the Factor VIII gene therapy program and the timing of a clinical trial of the product 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 preclinical studies and clinical trials; the content and timing of decisions by the U.S. Food and Drug Administration, the European Commission and other regulatory authorities concerning the program,; the ability to manufacture the product candidate 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 2011 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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