BioMarin Receives European Orphan Drug Designation for BMN 270, First Investigational AAV-Factor VIII Gene Therapy for Patients with Hemophilia A

SAN RAFAEL, Calif., March 24, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) announced today that BMN 270, an investigational gene therapy for the treatment of hemophilia A, has been granted orphan drug designation by the European Commission. In the European Union, orphan drug designation is given to treatments that are intended for life-threatening or chronically-debilitating conditions with a prevalence of not more than 5 in 10,000 people. Earlier this month, BioMarin announced BMN 270 had also received orphan drug designation from the U.S. Food and Drug Administration.

BMN 270 is an AAV 5 factor VIII vector designed to restore factor VIII plasma concentrations, essential for blood clotting in patients with hemophilia A. BioMarin is currently conducting a Phase 1/2 study to evaluate the safety and efficacy of BMN 270 gene therapy in patients with severe hemophilia A.

Study Design

The Phase 1/2 study will evaluate the safety and efficacy of BMN 270 gene therapy in up to 12 patients with severe hemophilia A. The primary endpoints are to assess the safety of a single intravenous administration of a recombinant AAV, human-coagulation factor VIII vector and to determine the change from baseline of factor VIII expression level at 16 weeks after infusion. The kinetics, duration and magnitude of AAV-mediated factor VIII activity in individuals with hemophilia A will be determined and correlated to an appropriate BMN 270 dose.

This is a dose escalation study with the goal of observing an increase in factor VIII levels. Secondary endpoints include assessing the impact of BMN 270 on the frequency of factor VIII replacement therapy, the number of bleeding episodes requiring treatment and any potential immune responses. Patients will be monitored for safety and durability of effect for five years.

About Hemophilia A

Hemophilia A, also called factor VIII (FVIII) deficiency or classic hemophilia, is a genetic disorder caused by missing or defective factor VIII, a clotting protein. Although it is passed down from parents to children, about 1/3 of cases are caused by a spontaneous mutation, a new mutation that was not inherited.[1] Approximately 1 in 10,000 people is born with hemophilia A.[2] People living with the disease are not able to form blood clots efficiently and are at risk for excessive bleeding from modest injuries, potentially endangering their life. People with severe hemophilia often bleed spontaneously into their muscles or joints. The standard of care for the 43 percent of hemophilia A patients, who are severely affected, is a prophylactic regimen of factor VIII infusions three times per week.[3] Even with prophylactic regimens, many patients still experience spontaneous bleeding events that result in progressive and debilitating joint damage.

About Gene Therapy

Gene therapy is a form of treatment designed to fix a genetic problem by adding a corrected copy of the defective gene. The functional gene is inserted into a vector — containing a small DNA sequence — that acts as a delivery mechanism, providing the ability to deliver the functional gene to cells. The cells can then use the information to build the functional proteins that the body needs, potentially reducing or eliminating the cause of the disease. Currently, gene therapy for the treatment of hemophilia A is available only as part of a clinical trial.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for patients with serious and life-threatening rare and ultra-rare genetic diseases. The company's portfolio consists of five commercialized products and multiple clinical and pre-clinical product candidates. 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 development of BioMarin's BMN 270 program generally and the timing and results of the clinical trial of BMN 270. 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 of BMN 270; 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 candidate 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 2015 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.

BioMarin® is a registered trademark of BioMarin Pharmaceutical Inc.

[1] Source: National Hemophilia Foundation
http://www.hemophilia.org/Bleeding-Disorders/Types-of-Bleeding-Disorders/Hemophilia-A

[2] Source: World Federation of Hemophilia
http://www.wfh.org/en/page.aspx?pid=637#How_common

[3] Source: World Federation of Hemophilia
http://www.wfh.org/en/resources/annual-global-survey
http://www.wfh.org/en/abd/prophylaxis/prophylaxis-administration-and-dosing-schedules

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