BioMarin Announces Acceptance of Late Breaking Abstract for BMN 270 at the World Federation of Hemophilia (WFH) 2016 World Congress July 27 in Orlando, FL

Investor event to be held in Orlando following the July 27 oral presentation at the WFH meeting

SAN RAFAEL, Calif., June 20, 2016 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (NASDAQ:BMRN) today announced the upcoming oral presentation summarizing a late breaking abstract accepted for the XXXII International Congress of the World Federation of Hemophilia. The presentation will report interim results of an open-label, Phase 1/2 study of BMN 270, an AAV5-FVIII Gene transfer in severe Hemophilia. BMN 270 has received orphan drug designation from the European Commission and U.S. Food and Drug Administration.

The data will be presented in the Late Breaking Gene Therapy session between 2:15pm-3:45pm ET in Room Hall A 4 by John Pasi, Professor of Haemostasis and Thrombosis, Barts and the London School of Medicine, Honorary Consultant Haematologist, The Royal London Hospital, a lead investigator on the study. The data presented at the WFH congress will be updated since the Company reported preliminary data April 20, 2016.

The WFH congress is being held July 24-28, 2016 in Orlando, Florida. A reprint of the abstract will be available following its presentation under the Investor Relations section of the BioMarin website at http://www.BMRN.com.

Following the oral presentation at the WFH congress on July 27 BioMarin management will host an investor event.

BMN 270 Study Design
The current Phase 1/2 study is evaluating 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 vector coding for human-coagulation factor VIII 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] As an X-linked disorder, hemophilia A mostly affects males, occurring in approximately 1 in 5,000 male births.[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 microbleeds and spontaneous bleeding events that result in progressive joint damage.

About Gene Therapy
Gene therapy is a 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. The AAV approach to gene therapy has been advanced at the University College London (UCL) in the treatment of hemophilia B. At UCL, this technology has shown evidence to be both safe and effective, correcting bleeding for greater than four years in a continuing clinical trial.

About BioMarin
BioMarin is a global biotechnology company that develops and commercializes innovative therapies for people with serious and life-threatening rare disorders. 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 pre-clinical studies and clinical trials of BMN 270; our ability to successfully manufacture the product candidate for the pre-clinical 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.

[1] Source: National Hemophilia Foundation
http://www.hemophilia.org/Bleeding-Disorders/Types-of-Bleeding-Disorders/Hemophilia-A
[2] Source: CDC
http://www.cdc.gov/ncbddd/hemophilia/data.html
[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

Contact:
Investors:
Traci McCarty
BioMarin Pharmaceutical Inc.
(415) 455-7558

Media:
Debra Charlesworth
BioMarin Pharmaceutical Inc.
(415) 455-7451
