BioMarin Announces Presentations at 59th American Society of Hematology Annual Meeting & Exposition

Oral Presentation to Include 78 Week Data with 6e13 Dose Cohort and 32 Week Data with 4e13 Dose Cohort from its Ongoing Phase 1/2 valoctocogene roxaparvovec (formerly BMN 270) Clinical Program, a Gene Therapy for Hemophilia A

SAN RAFAEL, Calif., Dec. 6, 2017 /PRNewswire/ -- BioMarin Pharmaceutical Inc. (NASDAQ: BMRN) announced today that the company will present data in a late breaking abstract session at the 59th American Society of Hematology (ASH) Annual Meeting & Exposition being held December 9-12, 2017 in Atlanta, Georgia. The presentation will report interim results from a Phase 1/2 study of valoctocogene roxaparvovec, an AAV5-FVIII Gene transfer in severe hemophilia.

Listing of Posters and Presentations Related to BioMarin Products and Programs at the 59th American Society of Hematology Annual Meeting & Exposition

Oral Presentation – Late Breaking Abstract Session

Title	Authors
Achievement of Normal Circulating Factor VIII Activity Following BMN 270 AAV5-FVIII Gene Transfer: Interim, Long-Term Efficacy and Safety Results from a Phase 1/2 Study in Patients with Severe Hemophilia A **Presentation:** December 11 at 7:30 AM	K. John Pasi, Savita Rangarajan, Benjamin Kim, Will Lester, David Perry, Bella Madan, Fatemeh Tavakkoli, Ke Yang, Glenn F. Pierce and Wing Yen Wong

Poster Presentations

Title	Authors
Impact of Pre-Existing Immunogenicity to AAV on Vector Transduction By BMN 270, an AAV5-Based Gene Therapy Treatment for Hemophilia A **Presentation:** December 10 at 6:00-8:00 PM **Poster/Presentation:** #3332	Brian Long, Krystal Sandza, Jennifer Holcomb, Juli Pherarolis, Lucy Crockett, Lillian Falese, Greg Hayes, Jeremy Arens, Charles A. O'Neill, Nancy Pryer, Carlos Fonck, Stephen Zoog and Christian Vettermann
Interim Analysis of Immunogenicity to the Vector Capsid and Transgene-Expressed Human FVIII in a Phase-1/2 Clinical Study of BMN 270, an AAV5-Mediated Gene Therapy for Hemophilia A **Presentation:** December 11 at 6:00-8:00 PM **Poster/Presentation:** #4611	Brian Long, Benjamin Kim, Wing Yen Wong, Ke Yang, Christian Vettermann, Nancy Pryer, Romain Hardet, Klaudia Kuranda, Philippe Veron, Federico Mingozzi, Glenn F. Pierce and Becky Schweighardt

About Hemophilia A

Hemophilia A is a genetic disease caused by the deficiency of clotting factor VIII. It is the most common type of hemophilia and occurs much more frequently in males; incidence is estimated to be 1 in 4,000-5,000 male births. People born with hemophilia produce little or no clotting factors. The two main types of hemophilia are A and B. People with hemophilia A are missing or have low levels of clotting factor VIII.

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 six

commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.biomarin.com.

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