BioMarin Hemophilia A Clinical Development Program: An Update for the Hemophilia Community

BioMarin is pleased to update the community regarding our ongoing gene therapy clinical trial program in hemophilia A. BioMarin's investigational gene therapy for hemophilia A has not been approved for use; it is in ongoing clinical trials evaluating its safety and efficacy.

Clinical Trial Overview

BioMarin's investigational gene therapy valoctocogene roxaparvovec, is currently being studied in adults with severe hemophilia A.

The first Phase 1/2 study was initiated in 2015 and involved 15 individuals and two dose levels. These individuals are now in long- term follow-up, post treatment with valoctocogene roxaparvovec.

The Phase 3 study (named GENEr8-1) is currently being conducted in 13 countries and is fully enrolled. 134 patients have received investigational gene therapy as part of this study.

On July 19, 2021 BioMarin provided an oral presentation on the ongoing Phase 3 pivotal study of valoctocogene roxaparvovec, including the most recent data of 1 year or more of follow up for individuals enrolled in the GENEr8-1 study. This update was provided at the International Society on Thrombosis and Haemostasis (ISTH) 2021 Virtual Congress.

BioMarin intends to present a five-year update from the Phase 1/2 study during an oral presentation at International Society on Thrombosis and Haemostasis (ISTH) 2021 Virtual Congress on Wednesday, July 21st, 2021.

BioMarin extends its sincere gratitude to all study participants who have helped make this milestone possible.

Regulatory Status:

In the United States, BioMarin plans to submit two-year follow-up safety and efficacy data on all study participants from the GENEr8-1 study to support the benefit/risk assessment of valoctocogene roxaparvovec. BioMarin is targeting a Biologics License Application (BLA) submission in the second quarter of 2022 assuming favorable study results, followed by an expected six-month review procedure by the FDA

In Europe, BioMarin resubmitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) on June 28, 2021. In May 2021, the EMA granted the company's request for accelerated assessment. Accelerated assessment reduces the time frame for the EMA Committee for Medicinal Products for Human Use (CHMP) and Committee for Advanced Therapies (CAT) to review a MAA for an Advanced Therapy Medicinal Product (ATMP). A CHMP opinion is anticipated in the first half of 2022.

For additional information:

- For information on BioMarin clinical studies, visit www.clinicaltrials.gov and type in the study code "BMN 270"
- For inquiries or to provide feedback from advocacy organisations, please contact patientadvocacy@bmrn.com
- Contact BioMarin Medical Information at medinfoeu@bmrn.com