Update for Hemophilia Associations

BioMarin is pleased to update the community regarding our 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 May 19, 2021 BioMarin released a high-level update on the Phase 1 / 2 study of valoctocogene roxaparvovec, including the most recent results based on 5 years of follow up data for individuals receiving the 6E13 vg/kg dose.

BioMarin intends to present more detail on the five-year update from the ongoing Phase 1 / 2 study during an oral presentation at the upcoming International Society on Thrombosis and Haemostasis (ISTH) 2021 Virtual Congress taking place July 17-21.

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 plans to submit a Marketing Authorization Application (MAA) for valoctocogene roxaparvovec for the treatment of severe hemophilia A with one-year results from the Phase 3 GENEr8-1 study to the European Medicines Agency (EMA) in June 2021.

For More Information:

- Visit www.clinicaltrials.gov and type in the study code BMN 270
- For inquiries or to provide feedback from advocacy organizations, please contact patientadvocacy@bmrn.com
- Contact BioMarin Medical Information at 1-800-983-4587 or medinfo@bmrn.com