Update for Hemophilia Associations

BioMarin is a global pharmaceutical company with more than 20 years of experience in developing medicines for rare genetic conditions.

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 May 31st 2020 BioMarin released an update on the Phase 1 / 2 study of valoctocogene roxaparvovec, including the most recent results based on 4 years of follow up data for individuals receiving the 6E13 vg/kg dose.

BioMarin intends to present a four-year update from the ongoing Phase 2 study in a late breaking oral presentation at World Federation of Hemophilia Virtual Summit on June 17, 2020.

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

Regulatory Status

The Food and Drug Administration (FDA) is reviewing the biologics license application, under Priority Review, for valoctocogene roxaparvovec with a PDUFA action date of August 21, 2020. The FDA priority review designation is granted to drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness of the treatment, prevention, or diagnosis of a serious condition.

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Regulatory Status (continued)

The European Medicines Agency (EMA) validated the Company's Marketing Authorization Application (MAA) for valoctocogene roxaparvovec which has been in review under accelerated assessment since January. Although the MAA remains under accelerated assessment at this time, the Company expects the review procedure to be extended by at least three months due to COVID-19 delays. Further, the Company believes there is a high possibility that the MAA will revert to the standard review procedure, as is the case with most filings that initially receive accelerated assessment. Because of the combination of these events, the Company expects an opinion from the Committee for Medicinal Products for Human Use (CHMP) in late 2020/early 2021.

The company believes that both submissions represent the first time a gene therapy product for any type of hemophilia indication is under review for marketing authorization by health authorities.

No gene therapies for hemophilia have been determined to be safe or effective or approved for use.

For additional 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 organizations, please contact patientadvocacy@bmrn.com
- Contact BioMarin Medical Information toll free at 1-800-983-4587 or medinfo@bmrn.com