Update for the Hemophilia Community

BioMarin's Biologics License Application (BLA) for valoctocogene roxaparvovec has been accepted for review by the U.S. Food and Drug Administration (FDA)

BioMarin is providing an update to the community regarding our ongoing gene therapy clinical trial program in hemophilia A. BioMarin's investigational gene therapy for severe hemophilia A has not been approved for use in the United States.

Clinical Trial Overview

BioMarin's 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. This study has been fully enrolled since November 2019 with134 participants who received investigational gene therapy. Participants in the study continue to be closely followed as part of this study.

BioMarin extends its sincere gratitude to our investigators and the study participants.

Regulatory Status

On September 29, BioMarin resubmitted a BLA to the FDA for its investigational gene therapy, valoctocogene roxaparvovec.

On October 12,2022, BioMarin's BLA for valoctocogene roxaparvovec was accepted for review by the US FDA with a review action date of March 2023. Subject to completion of the filing review, FDA evaluates the data provided by BioMarin and makes its decisions based on their assessment of safety and efficacy.

The FDA granted Regenerative Medicine Advanced Therapy (RMAT) designation to valoctocogene roxaparvovec in March 2021. RMAT is an expedited program intended to facilitate development and review of regenerative medicine therapies, such as valoctocogene roxaparvovec, that are expected to address an unmet medical need in patients with serious conditions. The RMAT designation is complementary to Breakthrough Therapy Designation, which the Company received for valoctocogene roxaparvovec in 2017.

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Regulatory Status (Continued)

In addition to the RMAT Designation and Breakthrough Therapy Designation, BioMarin's valoctocogene roxaparvovec also received orphan drug designation from the EMA and FDA for the treatment of severe hemophilia A. Orphan drug designation is reserved for medicines treating rare, life-threatening or chronically debilitating diseases.

BioMarin extends its sincere gratitude to our investigators and the study participants who have helped make this milestone possible.

For additional 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 medinfo@bmrn.com or toll free at 1 –800-983-4587