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

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 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 study participants have received investigational gene therapy as part of this study.

On May 31st, 2022 BioMarin released an update on the Phase 1/2 study of valoctocogene roxaparvovec, including the most recent results based on 6 years of follow up data for participants receiving the 6E13 vg/kg dose.

BioMarin intends to present a six-year update from the Phase 1/2 study during an oral presentation at the upcoming International Society on Thrombosis and Hemostasis (ISTH) 2022 Virtual Congress taking place July 9-13.

BioMarin extends its sincere gratitude to all study participants, families and care partners who have helped make this milestone possible.

Regulatory Status

In the United States, BioMarin plans to include the previously reported results from the two-year follow-up safety and efficacy data from the Phase 3 GENEr8-1 study in a Biologics Licensing Application (BLA) resubmission for valoctocogene roxaparvovec to the Food and Drug Administration (FDA). Based on recent feedback received from the FDA related to our plans for the upcoming BLA, the Agency has requested additional information and analyses of data to be included in the BLA prior to submission. The FDA has not requested additional pre-clinical or clinical studies. While at present, no requests have been made concerning evaluation of efficacy and safety three years after dosing from the GENEr8-1 study, we are aware that such data will become available during the anticipated BLA review. Based on these new information requests, the BLA resubmission is now expected by the end of September.

Regulatory Status (continued)

In Europe, the European Medicines Agency (EMA) continues the review of our Marketing Authorization Application (MAA) for valoctocogene roxaparvovec, and we anticipate a Committee for Medicinal Products for Human Use (CHMP) opinion mid-year 2022. BioMarin has provided the EMA with two-year follow-up safety and efficacy data from the GENEr8-1 study.

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

BioMarin Haemophilia A Clinical Development Programme: An Update for the Haemophilia Community

BioMarin is providing an update to the community regarding our ongoing gene therapy clinical trial program in haemophilia A. BioMarin's investigational gene therapy for haemophilia 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 haemophilia 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 study participants have received investigational gene therapy as part of this study.

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BioMarin intends to present a six-year update from the Phase 1/2 study during an oral presentation at the upcoming International Society on Thrombosis and Haemostasis (ISTH) 2022 Virtual Congress taking place July 9-13.

BioMarin extends its sincere gratitude to all study participants, families and care partners who have helped make this milestone possible.

Regulatory Status

In Europe, the European Medicines Agency (EMA) continues the review of our Marketing Authorization Application (MAA) for valoctocogene roxaparvovec, and we anticipate a Committee for Medicinal Products for Human Use (CHMP) opinion mid-year 2022. BioMarin has provided the EMA with two-year follow-up safety and efficacy data from the GENEr8-1 study.

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Regulatory Status (continued)

In the United States, BioMarin plans to include the previously reported results from the two-year follow-up safety and efficacy data from the Phase 3 GENEr8-1 study in a Biologics Licensing Application (BLA) resubmission for valoctocogene roxaparvovec to the Food and Drug Administration (FDA). Based on recent feedback received from the FDA related to our plans for the upcoming BLA, the Agency has requested additional information and analyses of data to be included in the BLA prior to submission. The FDA has not requested additional pre-clinical or clinical studies. While at present, no requests have been made concerning evaluation of efficacy and safety three years after dosing from the GENEr8-1 study, we are aware that such data will become available during the anticipated BLA review. Based on these new information requests, the BLA resubmission is now expected by the end of September.

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 medinfoeu@bmrn.com