Update for the Hemophilia Community

The European Medicines Agency grants conditional marketing approval (CMA) for valoctocogene roxaparvovec for the treatment of severe hemophilia A in adults without a history of inhibitors and without detectable antibodies to adeno-associated virus serotype 5 (AAV5)

BioMarin is providing an update to the community, as requested, 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; it is in ongoing clinical trials evaluating its safety and efficacy.

What is the European Medicines Agency (EMA)?

The EMA is responsible for the approval and regulation of medicines across all European Union member states and the European Economic Area (EEA). The EMA's role is to ensure that all medicines available on the EU market have acceptable efficacy and safety data. It is important to note that valoctocogene roxaparvovec has not been approved for use in the United States; it is in ongoing clinical trials evaluating its safety and efficacy.

What is a Conditional Marketing Approval (CMA)?

In Europe, a conditional marketing authorization may be granted in the interest of public health based on less comprehensive clinical data than normally required, where the benefit of immediate availability of the medicine outweighs the risk inherent in the fact that additional data are still required.

Continued

What is the US regulatory status for BioMarin's investigational gene therapy for severe hemophilia A?

In the United States, valoctocogene raxoparvovec has not been approved for use or determined to be safe or effective. 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. 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 270-301"
- 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