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

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.

Regulatory Status in the United States:

BioMarin intends 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, as previously requested by Food and Drug Administration (FDA). BioMarin is targeting a Biologics License Application (BLA) resubmission in the second quarter of 2022 assuming favorable study results, followed by an expected six-month review procedure by the FDA.

Clinical Development Program

BioMarin has multiple clinical studies underway in its comprehensive gene therapy program for the treatment of haemophilia A. In addition to the global Phase 3 study GENEr8-1 and the ongoing Phase 1/2 dose escalation study, the company is enrolling participants in a Phase 3b, single arm, open-label study to evaluate the efficacy and safety of valoctocogene roxaparvovec with prophylactic corticosteroids in people with severe hemophilia A. The company is running a Phase 1/2 Study with valoctocogene roxaparvovec in approximately 10 participants with pre-existing AAV5 antibodies, as well as another Phase 1/2 Study in people with haemophilia A with active or prior FVIII inhibitors.

Regulatory Status in Europe:

On June 28, 2021, BioMarin announced today that the company resubmitted a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for its investigational gene therapy, valoctocogene roxaparvovec, for adults with severe hemophilia A. In May 2021, the EMA granted the company's request for accelerated assessment. Accelerated assessment reduces the time frame for the EMA Committee for Medicinal Products for Human Use (CHMP) and Committee for Advanced Therapies (CAT) to review a MAA for an Advanced Therapy Medicinal Product (ATMP). A CHMP opinion is anticipated in the first half of 2022.

Applications are eligible for accelerated assessment if the CHMP and CAT decide the product is of major interest for public health, particularly from the point of view of therapeutic innovation. Evaluating an MAA under the EMA centralized procedure can take up to 210 days, not counting clock stops when applicants are requested to provide additional information. On request, the CHMP and CAT can reduce the time frame to 150 days if the applicant provides sufficient justification for an accelerated assessment, although an application initially designated for accelerated assessment can revert to the standard procedure during the review for a variety of reasons. The decision to grant accelerated assessment has no impact on the eventual CHMP and CAT opinion on whether a marketing authorization should be granted.

A patient's medical team remains the best source of information regarding any health effects of haemophilia A.

For additional information:

- For 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 at medinfoeu@bmrn.com

