A statement for the haemophilia community from BioMarin regarding a serious adverse event, deemed unrelated, by the Independent Data Safety Monitoring Committee, to the BioMarin haemophilia A gene therapy phase 1 / 2 trial (Clinical studies are ongoing)

BioMarin's investigational gene therapy for haemophilia A is currently being evaluated for safety and efficacy in ongoing clinical trials and is currently not approved for commercial use.

Clinical Trial Overview

BioMarin's investigational gene therapy for haemophilia A is currently being evaluated for safety and efficacy in ongoing clinical trials and is currently not approved for commercial use.

The safety of study participants is of paramount importance to BioMarin. In November 2021, BioMarin was notified in accordance with study procedures by a clinical trial investigator of a serious adverse event in an individual participating in a BioMarin haemophilia A clinical trial, which has been deemed unrelated to the therapy by the Independent Data Monitoring Committee (DMC). The study participant was part of an ongoing phase 1/2 study of our investigational gene therapy for haemophilia A also known as valoctocogene roxaparvovec. This study completed enrolment in 2017 and is ongoing. A serious adverse event (SAE) is the term used to describe the occurrence of a serious health issue in a study participant, regardless of whether it was caused by the treatment under investigation.

The participant noticed a lump in his neck that was later diagnosed as salivary gland cancer. He then reported it to the study team. The cancer was completely removed during surgery, and the individual is being closely followed by his personal health care team. Separately, he continues his monitoring associated with the clinical trial.

BioMarin applauds this person's close attention to his health and reporting to his physician, while participating in clinical research.

continued

Clinical Trial Overview (continued)

As with any serious adverse event, a committee of experts was quickly brought together to help determine the cause of the cancer and whether it may be related to the therapy being studied in the trial. The committee was composed of the BioMarin study team, the study investigator, the independent committee of experts that routinely monitors the study (Data Monitoring Committee (DMC)), as well as other medical and scientific experts. In this case, the event was deemed by the study team as well as external experts to be unrelated to the investigational gene therapy given the available information. The appropriate health authorities have been informed. In addition, further analyses are currently being carried out on the cancer tissue which was removed.

Patient safety is BioMarin's top priority, and we will continue to monitor this participant and his clinical course in order to assess and communicate any changes in the benefit-risk profile of the investigational treatment, or changes to study conduct that may be necessary, with input and guidance from the independent DMC.

This safety information was also shared publicly during an oral presentation during the European Association for Haemophilia and Allied Disorders (EAHAD) Congress held February 2-4, 2022.

All clinical studies with valoctocogene roxaparvovec remain active, including study enrollment.

We acknowledge and thank the members of this community for their continued commitment and for the huge contribution that they have made to research in haemophilia.

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

