Pfizer giroctocogene fitelparvovec (Hemophilia A gene therapy) AFFINE Phase 3 Study Update

November 5th, 2021

We would like to share a recent update to our gene therapy clinical program for Hemophilia A (C3731003; AFFINE study; https://clinicaltrials.gov/ct2/show/NCT04370054) evaluating giroctocogene fitelparvovec, that we are developing with Sangamo.

Following the observation of factor VIII (FVIII) levels greater than 150% in some study participants, and out of an abundance of caution, Pfizer voluntarily paused screening and dosing in our Phase 3 study to implement a change to our study protocol to provide clinical management guidance for elevated FVIII levels.

No thrombotic events have been seen in any patient treated with giroctocogene fitelparvovec to date and no adverse events thought to be associated with elevated FVIII levels have been observed. Following our decision to pause the study, the FDA informed Pfizer that the program was placed on clinical hold on November 3rd. Per FDA, further communication will provide us a fuller explanation of the FDA position in the near future..

Ensuring the safety of study participants is our first priority. All participants in the Phase 3 study are under close observation and are being carefully monitored for thrombotic events and FVIII activity levels, as per study protocol. Those participants with FVIII activity greater than 150% are being closely monitored and managed by the study investigators. Some have been treated with direct oral anticoagulants as a precautionary measure.

We are committed to resuming patient dosing in this phase 3 program and believe this gene therapy, if approved, could represent an important treatment option for patients with hemophilia A. We will submit the protocol amendment and associated documents to Health Authorities in the countries where the trial is being conducted and respond to the FDA clinical hold to obtain agreements to proceed. In addition, Ethics Review Boards approvals for the clinical management guidelines and other proposed changes will be obtained before the amendment will be implemented. We expect dosing will resume once these are completed. This will happen at different times for each site depending on local review timelines.

As always, we are grateful for the trust and partnership between Pfizer and the hemophilia community, and we assure you that we are working with the utmost care as we advance our program of gene therapy for hemophilia A.

Sincerely,
The Pfizer Hemophilia gene therapy team