



## **FDA places the Pfizer/Sangamo Therapeutics phase 3 AFFINE haemophilia A gene therapy study on clinical hold**

November 5, 2021 – On Tuesday, November 4, the U.S. Food and Drug Administration (FDA) placed the Pfizer/Sangamo haemophilia A gene therapy programme, including the pivotal phase 3 AFFINE study ([NCT04370054](https://clinicaltrials.gov/ct2/show/study/NCT04370054)), with giroctocogene fitelparvovec (SB-525 or PF-07055480), on clinical hold until the review of a proposed protocol amendment. ([Sangamo news release](#)).

The clinical hold was initiated in order to implement a protocol amendment following the observance of factor VIII levels greater than 150% in some participants in the trial. The protocol amendment is intended to provide guidelines for clinical management of elevated factor VIII levels.

The AFFINE trial is anticipated to be approximately 50 participants in total. More than 50% of the participants in the trial have received their dose of giroctocogene fitelparvovec. To date, no participant who has received this gene therapy has experienced a thrombotic event, and some participants are being treated with oral anticoagulants to reduce the risk of thrombosis. The pause has been reported to regulators, who will evaluate the protocol amendment and any re-initiating of additional dosing.

Unrelated to the pause of the haemophilia A gene therapy programme, Pfizer and the FDA decided not to perform an interim analysis for two haemophilia gene therapy trials, planning instead to wait for complete analyses on 50 or more participants in the haemophilia A trial and 40 in the haemophilia B trial. This decision will delay the final readout of the data from these two trials.

We are reporting this information, available in the public domain, and will continue to keep our patient communities informed of further relevant developments. If you have any questions, please contact your treating physician and haemophilia treatment centre.

### **About the AFFINE study**

The Phase 3 AFFINE (efficAcy and saFety Factor viii geNe thErapy in haemophilia A patients; NCT04370054) study is an open-label, multicentre, single-arm study to evaluate the efficacy and safety of a single infusion of giroctocogene fitelparvovec in more than 50 adult (ages 18-64 years) male participants with moderately severe to severe haemophilia A.



### **About giroctocogene fitelparvovec**

Giroctocogene fitelparvovec (SB-525 or PF-07055480) comprises a recombinant adeno-associated virus serotype 6 vector (AAV2/6) encoding the complementary deoxyribonucleic acid for B domain deleted human FVIII. The giroctocogene fitelparvovec expression cassette was designed for optimal liver-specific expression of FVIII protein and supports the production of high yields of the vector. The giroctocogene fitelparvovec transcriptional cassette incorporates multi-factorial modifications to the liver-specific promoter module, FVIII transgene, synthetic polyadenylation signal and vector backbone sequence.

### **Sources:**

1. Pfizer communication on 'Pfizer giroctocogene fitelparvovec (Hemophilia A gene therapy) AFFINE Phase 3 Study Update,' November 5, 2021.
2. Sangamo Therapeutics Reports Recent Business and Clinical Highlights and Third Quarter 2021 Financial Results, November 4, 2021 ([News Release](#))