Phase 3 Trial of Gene Therapy SB-525 for Hemophilia A on FDA Hold

by *Steve Bryson PhD* | November 10, 2021

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The U.S. Food and Drug Administration (FDA) has placed a clinical hold on the Phase 3 trial evaluating **SB-525** (giroctocogene fitelparvovec), an investigational gene therapy for **hemophilia A**.

This pause in study recruitment and dosing was taken to give the agency time to review changes to the AFFINE trial's protocol after unusually high factor levels were reported in some treated patients.

SB-525 is designed to deliver a healthy copy of the gene that encodes factor VIII, or FVIII — the blood-clotting protein that is missing or is defective in people with hemophilia A.

AFFINE (**NCT04370054**) is an open-label, multicenter Phase 3 study evaluating the efficacy and safety of a single infusion of SB-525 in about 50 men, ages 18–64, with moderate to severe hemophilia A.

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After being treated, some patients showed FVIII levels greater than 150%, and higher-than-usual factor levels raise a risk of blood clots. No adverse events attributed to these elevated levels have been observed, and some patients were given oral anticoagulants (blood thinners) as a "precautionary measure," **Pfizer** announced in a **study update**.

Pfizer, which is developing the therapy along with **Sangamo Therapeutics**, voluntarily paused patient screening and dosing to implement a change in the trial's protocol — the document describing a clinical study's goals, design and approaches — that gives

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erapy, if approved, could represent an important treatment option for patients with hemophilia A," Pfizer wrote in its communication statement.

More than half of enrolled patients have received SB-525 in the trial, and those with FVIII levels greater than 150% are being closely monitoring, the update stated.

"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 [blood clotting] events and FVIII activity levels, as per study protocol," Pfizer wrote.

The companies will also submit protocol amendments and associated documents to health authorities in other countries where the trial is being conducted. They expect dosing will resume, but when might depend on local review timelines.

Unrelated to SB-525's clinical hold and after discussions with the FDA, Pfizer decided not to perform an interim analysis for this trial, as well as for another Phase 3 trial (**NCT03861273**) assessing **SPK-9001** (also known as PF-06838435, fidanacogene elaparvovec), an experimental gene therapy for people with **hemophilia B** who lack the blood clotting protein factor IX (FIX).

Instead, the company will wait for a complete analysis of the 50 or more patients in the hemophilia A trial, and the 40 patients in the hemophilia B trial. Due to this decision, reporting of data from these two studies will be delayed.

Initially developed by Sangamo, SB-525 uses an adeno-associated viral vector (AAV6) to deliver genetic material to liver cells to allow the production of FVIII, with the aim of reducing or eliminating the need for a lifetime of **replacement therapies**. The AAV6 vector is engineered in a lab to be harmless and to specifically target liver cells.

This gene therapy is designed to be a one-time treatment administered into the bloodstream. SB-525 received orphan drug, fast track, and regenerative medicine advanced therapy designations in the U.S., as well as the designation of orphan medicinal product in Europe.

About the Author

Steve Bryson PhD Steve holds a PhD in Biochemistry from the Faculty of Medicine at the University of Toronto, Canada. He worked as a medical scientist for 18 years, within both industry and academia, where his research focused on the discovery of new medicines to treat inflammatory disorders and infectious diseases. Steve recently stepped away from the lab and into science communications, where he's helping make medical science information more accessible for everyone.

Tags

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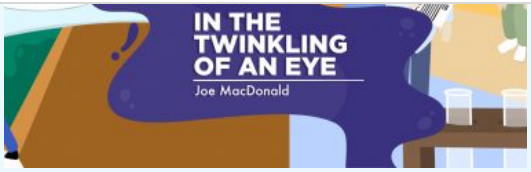

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