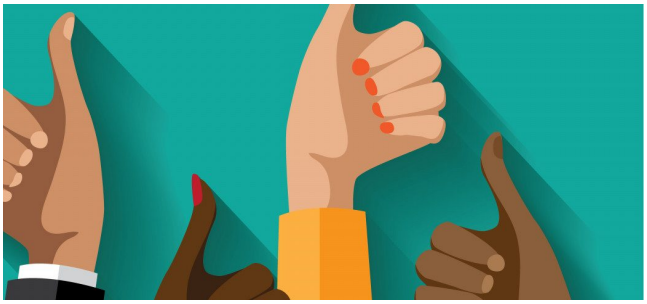
FDA Grants Sigilon Therapeutic's SIG-001 Orphan Drug Status for Hemophilia A

AUGUST 28, 2019 BY PATRICIA INACIO, PHD IN NEWS.



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The U.S. Food and Drug Administration (FDA) has granted orphan drug status to Sigilon Therapeutics' candidate cell therapy, called SIG-001, for hemophilia A.

Orphan drug status aims to encourage therapies for rare and serious diseases, through benefits such as seven years of market exclusivity and exemption from FDA application fees.

"We are very pleased to have received Orphan Drug Designation for SIG-001. The designation underscores the critical unmet need for effective, durable therapies for hemophilia A and reinforces our commitment to advance SIG-001 through our development program," Rogerio Vivaldi, MD, MBA, president and CEO of Sigilon, said in a press release.

"This is the first of what we hope will be multiple Orphan Drug Designations for Sigilon as we continue progressing novel therapies for chronic diseases through our pipeline," he added.

SIG-001 was developed using Sigilon's trademarked Shielded Living Therapeutics platform. The treatment candidate consists of human cells modified to incorporate large amounts of synthetic DNA-encoding therapeutic proteins into cells, specifically human FVIII.

Sigilon's cells were modified to have a shield made of synthetic biomaterial, called Afibromer. That shield blocks the triggering of the host's immune system, and prevents damaging reactions, like fibrosis (tissue scarring). Moreover, the biomaterial enhances the optimized delivery and long-term stability of the cells in the body.

With this new technology, the cells can promote long-term production of therapeutic proteins after a single intervention.

Preclinical results showed that delivering SIG-001 into the abdomen of a mouse model of hemophilia A led to a sustained production of FVIII, and effectively controlled the bleeding. The same benefits were seen in animal models of hemophilia B and factor VII deficiency, a rare clotting factor deficiency.

Researchers showed that delivering different doses of SIG-001 to the animal's abdomen led to a dose-depend secretion of therapeutic doses of FVIII. The treatment helped controlled bleeding time and blood loss during a bleeding test of the mouse tail. Moreover, the levels of FVIII remained stable for more than six months.

Sigilon is currently completing the work needed to support an investigational new drug (IND) application for SIG-001. The company expects to initiate the first clinical studies in patients with hemophilia A in 2020.