

Sangamo Therapeutics Logo

Sangamo Announces Treatment Of First Patient In Phase 1/2 Clinical Trial Of In Vivo Genome Editing Therapy For Hemophilia B

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RICHMOND, Calif., Dec. 17, 2018 /PRNewswire/ -- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomics medicines company, today announced treatment of the first patient in the Phase 1/2 clinical trial evaluating SB-FIX, an investigational *in vivo* genome editing therapy for patients with hemophilia B.



Hemophilia B is a rare, genetic bleeding disorder caused by a lack of the factor IX (FIX) protein, which is necessary for normal blood clotting. Current hemophilia B treatments typically require frequent intravenous infusions of clotting FIX to minimize the number of bleeding episodes, but the burden on the patients is high and a risk of bleeding is always possible. SB-FIX uses Sangamo's proprietary zinc finger nuclease (ZFN) genome editing to insert into the DNA of liver cells a corrective copy of the F9 gene, which controls the production of FIX. The goal of this therapy is to enable a patient's liver to produce a lifelong and stable supply of the clotting protein. Unlike conventional AAV cDNA gene therapy and lenti- or retroviral-based gene therapies that insert genes randomly into the genome, SB-FIX is designed with the goal to permanently and precisely integrate the F9 gene into the DNA.

"I am grateful to the first patient entering this study as we explore the symbolic step toward potentially changing what treatment could look like for patients with hemophilia B," said study investigator Craig Kessler, MD, a professor of oncology at Georgetown University. "This clinical trial will generate data highly anticipated by scientists, physicians and patients, and we are honored to be the first medical center to treat a patient." Georgetown is conducting the study with its clinical partner, MedStar Georgetown University Hospital, where Kessler directs the Center for Comprehensive Hemophilia and Thrombosis Care.

Sangamo's Phase 1/2 study is an open-label clinical trial designed to assess the safety, tolerability and preliminary efficacy of SB-FIX in adults with severe hemophilia B. The study is currently screening subjects in the United States at hospitals in Washington D.C., Duarte, Detroit, Indianapolis, Dallas, and in the United Kingdom in Glasgow, Birmingham and London.

"We are excited to begin to understand the potential of our *in vivo* gene editing technology for hemophilia B, which represents a completely new treatment approach for this disease," said Sangamo Chief Medical Officer, Edward Conner, MD. "We've made progress with patient recruitment in this clinical program and are hopeful for continued momentum in patient enrollment. We've also initiated clinical sites in the United Kingdom and are currently screening patients for enrollment into this study."

SB-FIX has received Orphan Drug and Fast Track designations from the U.S. Food and Drug Administration (FDA). Earlier this year, the Medicines and Healthcare Products Regulatory Agency (MHRA) granted the Clinical Trial Authorisation (CTA) for enrollment of patients in the UK into the ongoing Phase 1/2 clinical trial evaluating SB-FIX for hemophilia B. The CTA permits evaluation of SB-FIX in both adults and adolescents.

About Sangamo Therapeutics

Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic therapies with the potential to transform patients' lives using the Company's platform technologies in genome editing, gene therapy, gene regulation and cell therapy. For more information about Sangamo, visit www.sangamo.com.

Forward-Looking Statements

This press release may contain forward-looking statements based on Sangamo's current expectations. These forward-looking statements include, without limitation, references relating to the design of Sangamo's ZFN genome editing technology, designed with the goal to permanently and precisely integrate the F9 gene into the DNA, the potential of Sangamo's technology to treat hemophilia B by enabling a patient's liver to produce a lifelong and stable supply of the clotting protein, the potential to transform what treatment could look like for patients with hemophilia B, an accelerated rate of patient enrollment in the US and UK and the impact of Sangamo's clinical trials on the field of genomic medicine. Actual results may differ materially from these forward-looking statements due to a number of factors, including uncertainties relating to substantial dependence on the clinical success of lead therapeutic programs, the initiation and completion of stages of our clinical trials, whether the clinical trials will validate and support the tolerability and efficacy of ZFNs, technological challenges, ability to manufacture product candidates for our clinical trials, Sangamo's ability to develop commercially viable products and technological developments by our competitors. For a more detailed discussion of these and other risks, please see Sangamo's SEC filings, including the risk factors described in its most recent Quarterly Report on Form 10-Q. Sangamo assumes no obligation to update the forward-looking information contained in this press release.

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Investor Relations - United States, McDavid Stilwell, 510-970-6000, x219, mstilwell@sangamo.com, Varant Shirvanian, 510-970-6000 x205, vshirvanian@sangamo.com, Media Inquiries - United States, Aron Feingold, 510-970-6000, x421, afeingold@sangamo.com, Investor Relations and Media Inquiries - European Union, Caroline Courme, 33 4 97 21 27 27, ccourme@sangamo.com