

CIRM Funded Clinical Trials

A Phase 1/2 Study to Assess the Safety, Tolerability, and Efficacy of ST-400 Autologous HSPC Transplant in Transfusion-dependent β -Thalassemia

Disease Area:	Beta Thalassemia
Investigator:	Bettina Cockroft
Institution:	Sangamo BioSciences, Inc.
CIRM Grant:	CLIN2-11031
Award Value:	\$8,000,000
Trial Sponsor:	Sangamo BioSciences, Inc.
Trial Stage:	Phase 1/2
Trial Status:	Active, not recruiting
Targeted Enrollment:	6
ClinicalTrials.gov ID:	NCT03432364



Bettina Cockroft

Details:

Sangamo, Inc. is testing genetically engineered blood stem cells for the treatment of beta-thalassemia, a severe form of anemia caused by mutations in the hemoglobin gene. This genetic disorder requires life-long blood transfusions and carries a life expectancy of only 30-50 years. The Sangamo therapy takes a patient's own blood stem cells and, using a gene-editing technology called zinc finger nuclease (ZFN), provides a functional copy of the hemoglobin gene. These modified cells are given back to the patient which potentially will eliminate the need for chronic transfusions and its associated complications.

Design:

Open label, single arm study

Goal:

Safety and tolerability. Efficacy, change from baseline HbF levels, frequency and volume of RBC transfusions.

News about this clinical trial:

Sangamo Announces \$8 Million CIRM Grant For ST-400 -- A Gene-Edited Cell Therapy Candidate -- To Treat Transfusion-Dependent Bet