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

Grant Award Details

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

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-11031

Project Objective: Complete a Phase 1/2 clinical trial using Zinc-finger genome editing of the BCL11A enhancer at the gamma globin locus for treatment of beta thalassemia.

Investigator:

Name:	Bettina Cockroft
Institution:	Sangamo BioSciences, Inc.
Type:	PI

Disease Focus: Beta Thalassemia, Blood Disorders

Human Stem Cell Use: Adult Stem Cell

Cell Line Generation: Adult Stem Cell

Award Value: \$8,000,000

Status: Active

Grant Application Details

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

Public Abstract: **Therapeutic Candidate or Device**

ST-400 is a gene-edited cell therapy candidate for patients with transfusion-dependent beta-thalassemia

Indication

Transfusion-dependent beta-thalassemia

Therapeutic Mechanism

ST-400 is intended to disrupt BCL11A erythroid enhancer in CD34+ HSPC resulting in an increase in fetal hemoglobin which can substitute for reduced or absent adult Hb. Therefore, treatment with ST-400 may potentially reduce or eliminate need for chronic blood transfusions and likely improve quality of life of patients

Unmet Medical Need

Beta-thalassemia patients require life-long blood transfusions which result in iron overload in many organs. Consequently, patients require iron chelators to treat iron overload. Overall, thalassemia patients have lower quality of life and shorter lifespan compared to overall US population

Project Objective

Safety established, efficacy/activity observed

Major Proposed Activities

- Manufacture ST-400 for each subject in the proposed Phase 1 / 2 clinical study
- Assess safety, tolerability, biological activity, and clinical efficacy of ST-400 over a 52-week period
- Decision to continue development; plan Phase 3 study

Statement of Benefit to California:

About 200 beta-thalassemia patients are estimated to live in CA; ST-400 may be a useful for a substantial portion of these patients. Sangamo is an emerging California-based biotechnology company with about 170 employees in Richmond, CA. Two of the proposed clinical sites for the study are California based (Benioff Children's and UCLA). ST-400 will be manufactured by HCATS based in Mountain View, CA. Overall, this project will deliver significant medical and economic benefit to California

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