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**Curing Sickle cell Disease with CRISPR-Cas9 genome editing**

**Grant Award Details**

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Curing Sickle cell Disease with CRISPR-Cas9 genome editing

**Grant Type:** Late Stage Preclinical Projects

**Grant Number:** CLIN1-11497

**Project Objective:** File an IND application for a clinical trial using CRISPR-Cas9 genome editing of autologous HSC to treat sickle cell disease.

**Investigator:**

<b>Name:</b>	Mark Walters
<b>Institution:</b>	University of California, San Francisco
<b>Type:</b>	PI

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**Disease Focus:** Blood Disorders, Sickle Cell Disease

**Human Stem Cell Use:** Adult Stem Cell

**Award Value:** \$2,242,805

**Status:** Active

**Grant Application Details**

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**Application Title:** Curing Sickle cell Disease with CRISPR-Cas9 genome editing

**Public Abstract:****Therapeutic Candidate or Device**

Blood stem cells collected from individuals with sickle cell disease will have the sickle gene corrected and then given back to the same individual.

**Indication**

Sickle cell disease is a hereditary blood disorder associated with pain and other serious medical complications including a shortened life-span

**Therapeutic Mechanism**

It is possible to cure sickle cell disease by a bone marrow transplantation. Unfortunately, most patients do not have a donor for this treatment. In addition, a bone marrow transplant is a risky treatment. Our new treatment first collects a sickle cell person's own blood stem cells and uses a new technology called CRISPR to correct the sickle gene in the blood stem cells. These are returned to the same person after first destroying the sickle-producing blood cells. It might stop the disease.

**Unmet Medical Need**

Currently, there are only two approved treatments for sickle cell disease, which are drugs that help treat symptoms but do not cure the disorder. There is an unmet need to approve new treatments that eliminate the cause of the disorder that arises in the blood cells, with potential of cure.

**Project Objective**

Obtain an IND an early phase clinical trial

**Major Proposed Activities**

- Find all the sites in human DNA where the CRISPR changes the code and confirm these changes are not dangerous or cause cancer
- Find all the types of the hemoglobin protein that might be made after the CRISPR fixes the sickle gene and confirm the hemoglobin in red cells is safe
- make enough of the gene-corrected blood stem cells to treat 3 patients and show these are safe in mice and have a good shelf-life after freezing

**Statement of Benefit to California:**

Sickle cell disease, a hereditary blood disorder that primarily affects individuals of African descent, is estimated to affect more than 6000 persons in California. Most adults die of the disorder by their late 40s. A curative therapy given early in life would have a significant beneficial effect on lifespan and the quality of life, and reduce life-long healthcare costs to families and to society. The goal of this proposal is to offer better treatment for every person with the disorder.

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