A PHASE 1/2 CLINICAL TRIAL

CRISPR-SCD

Formal Trial Name

Transplantation of CRISPR-CAS9 Corrected Hematopoietic Stem Cells in Patients with Severe Sickle Cell Disease

Trial Funding

This study is funded in part by the National Institutes of Health (NIH)/National Heart, Lung, and Blood Institute (NHLBI) and the California Institute for Regenerative Medicine (CIRM).

Trial Purpose

To test whether the investigational product, CRISPR_SCD001:

- Can be made from people with sickle cell disease and if it is safe for them to receive it
- Improves the health of patients with sickle cell disease

Trial Background

The study is designed to test a new drug called CRISPR_SCD001, which is a type of gene editing therapy. Study doctors will use a 'gene changing' tool called CRISPR to change the sickle gene mutation in a person's own stem cells to a non-sickle gene that makes healthy hemoglobin. These gene edited stem cells will be given back to the patient, who is their own donor. Before giving back the gene edited cells, patients will undergo a procedure that first destroys the sickle-producing stem cells. The procedure is done after first giving high-dose chemotherapy to make room for the gene edited stem cells to grow. The safety of making this change to their stem cells will be tested and monitored so study doctors can find out if gene editing is safe, and if this treatment might help people with severe sickle cell disease get better.

"This therapy has the potential to transform sickle cell disease care by producing an accessible, curative treatment that is safer than the current therapy of stem cell transplant from a healthy bone marrow donor. If this is successfully applied in young patients, it has the potential to prevent irreversible complications of the disease."

-Mark Walters, MD

TRIAL INFORMATION

Eligibility

- **Male or Female with Hemoglobin SS**
- **12-35 years old**

HAVE SIGNS OF SEVERE SICKLE CELL DISEASE, INCLUDING:

- **Repeated episodes of severe pain, including at least four severe vaso-occlusive pain events in the last two years.**
- **History of two or more episodes of acute chest syndrome in the last two years.**

ADEQUATE PHYSICAL FUNCTION, INCLUDING:

- **Being able to care for oneself.**
- **Good heart, lung, kidney and liver function.**
- **No extensive liver damage from excess iron.**

Patients who are pregnant or breastfeeding, have already received a bone marrow or solid organ transplant, had a stroke or are receiving treatment to prevent a stroke, or participated in another clinical trial within the past three months are not eligible to participate in the trial.
Additional information on inclusion and exclusion criteria can be provided by the trial team. See "for more information" contact below.

PARTICIPANTS IN THIS STUDY WILL:

- **Be evaluated at a study site in California to assess their ability to be part of the trial.**
- **Have monthly blood transfusions for at least two months prior to gene therapy to prepare for the treatment.**
- **Undergo collection of their blood stem cells for gene editing therapy.**
- **Be admitted to a hematopoietic stem cell transplant inpatient unit at a study site for at leastone month for bone marrow conditioning, gene therapy administration, and recovery**
- **Undergo close monitoring after gene editing therapy to assess the safety and efficacy of thetreatment.**

Trial Sites

- **University of California San Francisco (UCSF), Benioff Children's Hospital, Oakland**
- **University of California, Los Angeles (UCLA)**

Contact

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Download Trial Details

POTENTIAL QUESTIONS FOR PATIENTS TO DISCUSS WITH PROVIDERS AND THE TRIAL SITE

- **Do you think this trial could be an option for me to consider? Why?**
- **Would I be eligible to participate based on my medical history?**
- **What are the risks? What are the benefits?**
- **What will happen if I decide to participate?**
- **Will my care be any different if I choose to participate?**
- **What will be the role of my provider while I am participating?**
- **How long is the trial process?**
- **How long will I be in the hospital?**
- **What specifically will happen during the trial? What procedures will be done? What are the side effects?**
- **What happens after the trial?**

TRIAL-SPECIFIC LINKS

- Revolutionary Care: An Oakland Story Podcast
- UC Consortium Launches First Clinical Trial Using CRISPR
- Launch of CRISPR sickle cell trial
- Hope for a Cure