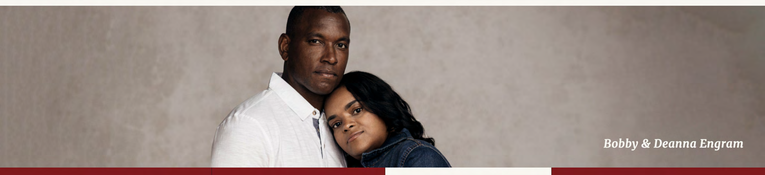

Bobby & Deanna Engram

At the Initiative, we believe clinical trial participation is one of the most important ways we can move towards a future without sickle cell disease.

About Clinical Trials

Clinical trials are medical studies that help find safe and effective ways to prevent, detect, or treat diseases. The Cure Sickle Cell Initiative (CureSCi) is working with researchers across the country to move promising genetic therapies into clinical trials.

CureSCi clinical trials focus first on the safety of the new treatment (Phase I). In a later stage clinical trial (Phase II), the same treatment is tested to see if it can cure or severely reduce the severity of sickle cell disease. Each phase of the clinical trial will answer important questions about safety and effectiveness.

The Phase I and Phase II genetic therapy clinical trials listed on this page can help us find a path to improving the lives of those living with sickle cell disease.

Ways You Can Help

One of the most significant ways to support the Cure Sickle Cell Initiative is to learn more about the importance of clinical trials and to make an informed decision about participating, when trials are available. How to overcome barriers to progress, which includes exploring beliefs about clinical trials participation, is an important conversation in the sickle cell disease community.

CureSCi Supported Clinical Trials

Although genetic therapies are a promising option for many rare diseases, the technique is still being studied to make sure that it is safe and effective. The National Heart, Lung, and Blood Institute, part of the National Institutes of Health, is partnering with researchers through CureSCi to design and fund genetic therapy clinical trials. These trials are investigational, and treatments are not currently approved by the U.S. Food and Drug Administration (FDA).

One of the most significant ways to support cures for sickle cell disease is to learn about the importance of clinical trials and make an informed decision about participating.

CURRENT CLINICAL TRIALS

TRIAL PHASE 2

GRASP

Gene therapy to Reduce All Sickle Pain
Funded in part by the National Heart, Lung, and Blood Institute and the California Institute for Regenerative Medicine

VIEW CLINICAL TRIAL OVERVIEW

TRIAL PHASE 1/2

Transplantation of CRISPR-CAS9 Corrected Hematopoietic Stem Cells (CRISPR_SCD-001) in Patients with Severe Sickle Cell Disease

VIEW CLINICAL TRIAL OVERVIEW

U.S. Food & Drug Administration: Clinical Trials- What Patients Need to Know

ASH Research Collaborative: Why Participate in Clinical Trials for Sickle Cell Disease?

EN ESPAÑOL

Cure Sickle Cell Initiative Patient Brochure

FROM CURESCI

Cure Sickle Cell Initiative Patient Brochure

CISCRP Participant Bill of Rights

CISCRP Questions to Ask

RESOURCES FOR FINDING SICKLE CELL CLINICAL TRIALS

FROM CURESCI

Clinical Trials Listing Handout

ClinicalTrials.gov

RESOURCE LIBRARY

A PHASE 2 CLINICAL TRIAL

GRASP

Gene therapy to Reduce All Sickle Pain

Full Trial Name

A Gene Transfer Study Inducing Fetal Hemoglobin in Sickle Cell Disease

Trial Funding

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

Trial Purpose

To test whether the gene therapy approach (lentiviral vector shRNA) which decreases expression of the BCL11A gene can improve or eliminate painful episodes.

Trial Background

This phase 2 trial is based on a pilot/phase 1 study also funded by the NIH being completed at Boston Children's Hospital, which showed early safety and efficacy in the initial 10 patients enrolled in the trial.

The study is designed to test whether a gene therapy approach targeting the BCL11A gene can eliminate painful episodes. In this study, a patients' blood-forming cells (blood stem cells) are collected and modified in the laboratory (gene transfer) in order to reduce the expression of the form of hemoglobin that causes the red blood cells to sickle and to increase a naturally occurring form of hemoglobin that does not sickle called fetal hemoglobin (HbF). The goal of this gene therapy is to maintain high levels of HbF production. In a pilot study, decreasing the expression of this gene in sickle cell patients increased the amount of fetal hemoglobin while also reducing the amount of sickle hemoglobin in their blood, which reduced the severity of the disease. The gene being targeted for change in this study controls the level of fetal hemoglobin is BCL11A (B cell lymphoma/leukemia 11A) that is not required for red blood cells to function.

"We know that just after birth babies with sickle cell disease have few symptoms due to their high levels of fetal hemoglobin but that their red blood cells function perfectly. We aim to "flip the switch" back in red cells by changing the adult red cells into cells more like those in a baby."

-David A. Williams, MD

TRIAL INFORMATION

Eligibility

- Male or Female
- 13–40 years old
- Had four or more pain episodes or acute chest syndrome requiring hospitalizations or clinic or emergency room visits in the past 2 years.

PARTICIPANTS IN THIS STUDY WILL:

- Be evaluated at a study site (see below for site listing) to assess their ability to be part of the trial.
- Have monthly blood transfusions for 3 months prior to gene therapy to prepare for the treatment.
- Undergo collection of their blood stem cells for gene therapy.
- Be admitted to a hematopoietic stem cell transplant inpatient unit at a study site for at least one month for bone marrow conditioning, gene therapy administration, and recovery.
- Undergo close monitoring after gene therapy to assess the safety and efficacy of the treatment.

Trial Sites

- Ann & Robert H. Lurie Children's Hospital of Chicago, Chicago, IL
- Boston Children's Hospital, Boston, MA
- Children's Hospital of Los Angeles, Los Angeles, CA
- Children's National Medical Center, Washington, DC
- Dana-Farber Cancer Institute/Brigham & Women's Hospital, Boston, MA
- Emory University, Atlanta, GA
- Medical College of Wisconsin, Milwaukee, WI
- University of California Davis, CA
- University of California Los Angeles (UCLA), Los Angeles, CA
- University of California San Francisco (UCSF), Benioff Children's Hospital of Oakland, Oakland, CA

Contact

FOR MORE INFORMATION
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Boston, MA 02115

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E-mail: dawilliams@childrens.harvard.edu

Clinical Trial Listing (Clinicaltrials.gov)

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

NEJM: Post-Transcriptional Genetic Silencing of BCL11A to Treat Sickle Cell Disease