CURE
SICKLE
CELL.

CURRENT & ONGOING GENE THERAPY CLINICAL TRIALS IN SICKLE CELL DISEASE

It's time to rewrite the story of sickle cell.

Clinical trials are research studies that look for safe and effective ways to prevent, find, or treat diseases. Some trials are exploring gene therapies, which involve changing a person's genetic material to treat a disease. This often means adding a healthy version of a gene that is not working properly in the patient's cells. This approach can help treat a disease without needing drugs or surgery. Researchers are also testing other methods, such as turning off genes that are causing problems or correcting a defective gene to help fight diseases. In 2023, the FDA approved two gene therapies for sickle cell disease (SCD), showing that these treatments are now a real option for people living with the disease. The NHLBI and other organizations continue to conduct trials to test gene therapies for SCD.

How can you help rewrite the story of sickle cell disease? Clinical trial participation is one of the most important ways we can move towards a future without sickle cell disease. We encourage adults, as well as children, healthy volunteers, those living with sickle cell disease, and people from various ethnic and racial backgrounds to consider participating in clinical trials.

Researchers are currently studying a number of potential new treatment options. This document includes gene-therapy trials. For information on other sickle cell disease trials, please visit: *https://clinicaltrials.gov*.

Have questions and want to learn more about clinical trials? Go to www.nih.gov/health-information/nih-clinical-research-trials-you.

National Heart, Lung, and Blood Institute

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INTERVENTIONAL

▸ Transplantation of CRISPRCas9 Corrected Hematopoietic Stem Cells (CRISPR_SCD001) in Patients With Severe Sickle Cell Disease: *Phase 1 / 2* *

RECRUITING

Age Range	**12–35 Years**
Trial Time Frame	**9/2024–3/2029**
Ref. No.	**NCT04774536**

This is an open label, non-randomized, 2-center, phase 1/2 trial of a single infusion of sickle allele modified cluster of differentiation (CD34+) hematopoietic stem progenitor cells (HSPCs) in subjects with in subjects ≥12 years old to 35 years old severe Sickle Cell Disease (SCD). The study will evaluate the hematopoietic stem cell transplantation (HSCT) using CRISPR/Cas9 edited red blood cells (known as CRISPR_SCD001 Drug Product).

https://clinicaltrials.gov/ct2/show/NCT04774536

▸ A Study Evaluating the Safety and Efficacy of BEAM-101 in Patients With Severe Sickle Cell Disease (BEACON): *Phase 1/2*

RECRUITING

Age Range	**18–35 Years**
Trial Time Frame	**8/2022–2/2025**
Ref. No.	**NCT05456880**

This is an open-label, single-arm, multicenter, Phase 1/2 study evaluating the safety and efficacy of the administration of autologous base edited CD34+ HSPCs (BEAM-101) in patients with severe SCD.

https://clinicaltrials.gov/ct2/show/NCT05456880

▸ Evaluation of Efficacy and Safety of a Single Dose of CTX001 in Participants With Transfusion-Dependent β-Thalassemia and Severe Sickle Cell Disease: *Phase 3*

RECRUITING

Age Range	**12–25 Years**
Trial Time Frame	**8/2022–6/2027**
Ref. No.	**NCT05477563**

This is a single-dose, open-label study in participants with transfusion-dependent β-thalassemia (TDT) or severe SCD. The study will evaluate the safety and efficacy of autologous CRISPR-Cas9 modified CD34+ human hematopoietic stem and progenitor cells (hHSPCs) using CTX001.

https://clinicaltrials.gov/ct2/show/NCT05477563

▸ St. Jude Autologous Genome Edited Stem Cells For Sickle Cell Disease-1 (SAGES1): *Phase 1*

RECRUITING

Age Range	**18–24 Years**
Trial Time Frame	**3/2025–12/2032**
Ref. No.	**NCT06506461**

This study is being done to test the safety of a new treatment called gene editing in Sickle Cell Disease (SCD) patients and to see if a single dose of this genetically modified cellular product will increase the amount of a certain hemoglobin called fetal hemoglobin (HbF) and help reduce the symptoms of SCD.

https://clinicaltrials.gov/ct2/show/NCT06506461

▸ Gene Correction in Autologous CD34+ Hematopoietic Stem Cells (HbS to HbA) to Treat Severe Sickle Cell Disease (Restore): *Phase 1/2*

RECRUITING

Age Range	**12-40 Years**
Trial Time Frame	**11/2021–12/2028**
Ref. No.	**NCT04819841**

This study is a first-in-human, single-arm, open-label Phase I/II study of nula-cel in approximately 15 participants, diagnosed with severe Sickle Cell Disease. The primary objective is to evaluate safety of the treatment in this patient population, as well as preliminary efficacy and pharmacodynamic data.

https://clinicaltrials.gov/ct2/show/NCT04819841

* *Funded by the Cure Sickle Cell Initiative*

OBSERVATIONAL

▸ Cooperative Assessment of Late Effects for SCD Curative Therapies (COALESCE)

RECRUITING

Age Range	**4–65 Years**
Trial Time Frame	**7/2022–2/2026**
Ref. No.	**NCT05153967**

The primary goal of this study is to determine whether curative therapies for individuals with SCD will result in improved or worsening heart, lung, and kidney damage when compared to individuals with SCD receiving standard therapy. The investigators will also explore whether certain genes are associated with a good or bad outcome after curative therapy for SCD.

https://ClinicalTrials.gov/show/NCT05153967

▸ Discarded Bone Marrow for Hematology Research

RECRUITING

Age Range	**Child, Adult, Older Adult**
Trial Time Frame	**7/2022–1/2035**
Ref. No.	**NCT04671212**

The primary objective of this study is to establish a mechanism to obtain discarded bone marrow-containing bone samples from hemoglobinopathy, as well as non-hemoglobinopathy individuals. The processing of samples will help to understand how best to manipulate HSPC's from hemoglobinopathy patients with gene therapy and gene technologies in the laboratory environment. It will also allow us to establish a reservoir of samples that can be studied in the future to assess cellular function and fitness for transplant.

https://ClinicalTrials.gov/show/NCT04671212

▸ Long-term Follow-up (LTFU) of Patients Treated With Genome-edited Autologous Hematopoietic Stem and Progenitor Cells (HSPC)

RECRUITING

Age Range	**18 Years and Older**
Trial Time Frame	**4/2024–1/2039**
Ref. No.	**NCT06155500**

This study is monitoring patients treated with OTQ923, an investigational drug product of ex vivo genome-edited autologous hematopoietic stem and progenitor cells (HSPCs) that induces fetal hemoglobin (HbF) production, for a total of 15 years following infusion to monitor long-term safety and efficacy.

https://www.clinicaltrials.gov/study/NCT06155500