

CURE SICKLE CELL.



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

OVER THE YEARS, much progress has been made in the effort to treat and prevent complications of sickle cell disease (SCD), but challenges remain for those living with the disease. Now, because of developments in the field of genetic therapies, there is increasing hope for a cure. To accelerate those developments, the Cure Sickle Cell Initiative is bringing together patients, caregivers, advocates, academic and industry researchers, and federal partners. Please join us on this exciting journey.

BUILDING ON NHLBI'S LEGACY OF EXCELLENCE IN SICKLE CELL DISEASE (SCD) RESEARCH

1948

NHLBI-funded research helped discover how SCD is inherited.

1972

Law established support for SCD screening, counseling, education, and research training.

1977

Study helped understand growth, development, pain, and complications in patients who have SCD.

1986

Landmark NHLBI-funded study showed an antibiotic could prevent deadly bacterial infections in children who have SCD.

1987

An NHLBI-hosted panel of experts recommended newborn screening for SCD.

1995

Landmark NHLBI-funded study showed hydroxyurea reduced pain crises, acute chest syndrome, hospitalizations, and transfusions.

1997

NHLBI-funded STOP study found screening methods and continuous transfusions lowered the risk of stroke in certain patients who have SCD.

1998

Based on NHLBI-funded research, the FDA approved hydroxyurea to prevent pain crises.

2001

NHLBI-funded research led to improved ways to perform blood and bone marrow transplants in children who have SCD.

2005

NHLBI-funded STOP II study found that stopping blood transfusions increased the risk of stroke in children who have SCD.

2009

NHLBI study found chronic pain is common in patients who have SCD.

NHLBI research led to improved ways to perform blood and bone marrow transplants in adults who have SCD.

2010

Federal partnership formed to help understand how common SCD is in the United States.

2011

NHLBI-funded BABY HUG study found hydroxyurea to be safe for young children who have SCD.

2014

NHLBI published SCD Expert Panel Report to help patients receive appropriate care.

NHLBI-funded researchers used gene editing to correct hemoglobin S gene in the laboratory for the first time.

2015

The NHLBI launched a new effort to help understand and overcome barriers to care for patients who have SCD.

2016

NHLBI-funded study found hydroxyurea as effective as blood transfusions at reducing risk factors for stroke in children who have SCD.

2018 & Beyond

NHLBI-led *Cure Sickle Cell* Initiative launched to accelerate development of genetic therapies to cure SCD. The Initiative complements other NHLBI research on sickle cell disease.



FREQUENTLY ASKED QUESTIONS

WHAT ARE THE GOALS OF THE CURE SICKLE CELL INITIATIVE?

The Initiative aims to:

- Create a collaborative, patient-focused research environment.
- Engage academic researchers, private sector researchers, advocates, patients, and caregivers to develop strategies for cures.
- Determine the safest, most effective, and most readily and widely adoptable genetic therapies.
- Move newly developed genetic therapies, including gene-editing approaches, into clinical trials.

HOW DOES THE INITIATIVE DIFFER FROM OTHER EFFORTS IN SICKLE CELL DISEASE?

The *Cure Sickle Cell* Initiative was developed to identify and support the most promising genetic cures for sickle cell disease. It is different from other efforts because it builds on the growing number of technological advancements and latest discoveries in genetics to help speed up our drive toward cures. The Initiative and its research partners are working to establish a national data warehouse of genetic therapies for SCD. They also are looking at different curative approaches to assess both clinical and cost effectiveness of each.

The Initiative recognizes the critical voices of patients, advocates, and caregivers, and every aspect of this effort is informed by those affected by SCD.

HOW WILL PEOPLE LIVING WITH SCD BE INVOLVED IN THE INITIATIVE? WILL CHILDREN BE INCLUDED?

We recognize the vital role of people living with SCD in our efforts to find new cures. Their engagement is a critical component of the Initiative, which is why they will work alongside researchers not only to help set the research agenda, but also help find ways to educate and

recruit patients to participate in clinical trials. Patient representatives already are involved at every level of the Initiative, and we continue to attend advocacy group meetings and conduct listening sessions and focus groups in the community to help guide the Initiative's efforts.

Genetic therapies that are being studied as a potential cure for SCD will require testing. Our initial efforts are focused on determining safety in adults, and once established—and if approved by the Food and Drug Administration (FDA)—we hope to begin trials that involve the participation of children and adolescents.

WHEN MIGHT NEW TREATMENTS OR A CURE BE AVAILABLE TO PEOPLE LIVING WITH SCD?

NIH and NHLBI are funding research and collaborating with researchers to move potential genetic therapies safely into clinical trials and these trials will be focused on people living with sickle cell disease. If studies meet the FDA requirements regarding safety and efficacy, then larger trials with even more patients will be developed.

HOW CAN I SUPPORT THE EFFORT?

Over the years, patients have been the cornerstone of NHLBI's research, and the *Cure Sickle Cell* Initiative is no different. Our combined efforts depend on the inputs, opinions, and suggestions from our diverse stakeholder groups, but most essentially from those living with SCD. We cannot seize scientific opportunities and advance cures without the full support and engagement of the patient community.

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

**To learn more about clinical trials,
visit:** <https://www.nih.gov/health-information/nih-clinical-research-trials-you>

**If you would like to learn more, please
reach out through the “Contact” link
on:** www.curesickle.org.

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