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**CURE
SICKLE
CELL.**



National Heart, Lung,
and Blood Institute

CURE SICKLE CELL.



Rewriting 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 gene therapies, there is increasing hope for safe and effective new treatment approaches that will transform the lives of people who have SCD. To accelerate those developments, the Cure Sickle Cell Initiative has brought 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 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 or stroke in children who have SCD.

2018

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

2019

The FDA approved voxelotor (removed from market in 2024) to prevent sickling of red blood cells and crizanlizumab-tmca to reduce vaso-occlusive crises.

2020

Cure Sickle Cell Initiative co-funds Phase 1 and Phase 2 SCD gene therapy trials.

2023

The FDA approved the first cell-based gene therapies, Casgevy and Lyfgenia, for the treatment of 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.
- Determine the safest, most effective, and most readily and widely adoptable gene therapies for SCD.
- Move newly developed gene 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 gene therapies 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 transform the lives of people with SCD.

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 HAVE PEOPLE LIVING WITH SCD BEEN INVOLVED IN THE INITIATIVE?

We recognize the vital role of people living with SCD in our efforts. Their engagement is a critical component of the Initiative, which is why they have worked alongside researchers not only to help set the research agenda, but also to help find ways to educate and recruit patients to participate in clinical trials. Patient representatives have been involved at every level of the Initiative, and they continue to guide efforts.

WHEN WILL NEW TREATMENTS BE AVAILABLE TO PEOPLE LIVING WITH SCD?

In December 2023, the FDA approved two new therapies, the first cell-based gene therapies for the treatment of sickle cell disease. NIH and NHLBI continue to fund research and collaborate with researchers to move potential gene therapies safely into clinical trials.

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.

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