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

We will be sharing information soon about new opportunities for patient engagement.

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

NATIONWIDE OUTREACH

We’re listening and we’re learning. We’ve started to travel around the country hearing about the experiences of people living with sickle cell disease. Below is a map of where we’ve been, and we look forward to expanding our presence to better understand the needs of the SCD community.

If you are interested in learning more about the Cure Sickle Cell Initiative, please visit our website at curesickle.org

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.

CURE SICKLE CELL.

Version 2.1; Updated: 18 May 2020
NHLBI-funded research helped discover how SCD is inherited.

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

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

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

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

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

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

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

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

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

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

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

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.

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

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

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.

**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 research within five to 10 years.

**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 strategies for cures.

**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 within five to 10 years, 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.