# Building on Our Legacy of Excellence in Sickle Cell Disease Research

NIH National Heart, Lung, and Blood Institute



• NHLBI-funded research helped discover how sickle cell disease is inherited. • Law established support for sickle cell disease screening, counseling, education, and research training.

#### 1972

### 1987

• An NHLBI-hosted panel of experts recommended newborn screening for sickle cell disease.

#### 1986

• Landmark NHLBIfunded study showed an antibiotic could prevent deadly bacterial infections in children who have sickle cell disease.

#### 1977

• Study helped understand growth, development, pain, and complications in patients who have sickle cell disease.

 Landmark NHLBIfunded study showed hydroxyurea reduced pain crises, acute chest syndrome, hospitalizations, and transfusions.

### 1995

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

1997

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

## 2009

- NHLBI study found chronic pain is common in patients who have sickle cell disease.
- NHLBI research led to improved ways to perform blood and bone marrow transplants in adults who have sickle cell disease.

### 2005

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

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### 2001

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

- Federal partnership formed to help understand how common sickle cell disease is in the United States.
- NHLBI-funded BABY HUG study found hydroxyurea to be safe for young children who have sickle cell disease.





- NHLBI published sickle cell disease 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 sickle cell disease.

### 2018 and Beyond

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

#### 2016

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

sicklecell.nhlbi.nih.gov