

**Thanks to recent advancements in gene therapy and decades of NIH-funded research, sickle cell disease (SCD) is now the first common genetic disorder to have been cured in some patients.**

## Sickle Cell Disease

is an inherited, lifelong blood disorder that affects red blood cells.

**Normal red blood cells are round,** which allows them to pass through small blood vessels and deliver oxygen throughout the body.



**SDC causes red blood cells to form into a crescent shape,** like a sickle.



Sickle-shaped red blood cells clump together, stick to vessel walls, and block normal blood flow, **resulting in a variety of serious health consequences.**

**~100,000** Americans **have SCD.**

**~3,000,000** Americans **carry the sickle cell genetic trait.**

**1 in 365** African-Americans **are born** with SCD.

Sickle Cell Coalition. Understanding the Impact of Sickle Cell Disease. (2019)

*"It's an exhilarating success story for those of us who have waited and hoped for this day."*

Dr. Francis Collins, Director, National Institutes of Health  
"At 16, She's a Pioneer in the Fight to Cure Sickle Cell Disease" (New York Times. Jan. 11, 2020)

The National Institutes of Health (NIH) **invested \$139 million in 2019 on research aimed at finding better treatments and cures** for SCD.

In 2018, the National Heart, Lung, and Blood Institute (NHLBI) launched the **Cure Sickle Cell Initiative**, which leverages the latest genetic discoveries and technological advances to **test promising and potentially curative gene therapies for SCD in clinical trials.**

For patients like 16-year-old Helen Obando, **these clinical trials are already paying off**, eliminating signs of SCD from their bodies.



Continued success against SCD - one of the thousands of genetic disorders with a known DNA glitch - could have far-reaching implications for the treatment of other rare diseases.



Sources: National Heart, Lung, and Blood Institute. (2019). Retrieved from [nhlbi.nih.gov/health-topics/sickle-cell-disease](https://nhlbi.nih.gov/health-topics/sickle-cell-disease); National Institutes of Health (2018). Retrieved from [nih.gov/news-events/news-releases/nih-launches-initiative-accelerate-genetic-therapies-cure-sickle-cell-disease](https://nih.gov/news-events/news-releases/nih-launches-initiative-accelerate-genetic-therapies-cure-sickle-cell-disease); New York Times. Retrieved from [nytimes.com/2020/01/11/health/sickle-cell-disease-cure](https://nytimes.com/2020/01/11/health/sickle-cell-disease-cure); National Institutes of Health (2020). Retrieved from [report.nih.gov/categorical\\_spending.aspx](https://report.nih.gov/categorical_spending.aspx)

New SCD treatments are helping patients live longer, better lives. But continued progress - and relief for those affected - depends on NIH funding growing reliably every year.