SICKLE CELL DISEASE

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.


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.

“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)

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.


ActforNIH.org | 202.465.8710 | connect@ACTforNIH.org | @ACTforNIH