RESEARCH AMERICA
DISCOVERY. INNOVATION. HEALTH.

If you think research is expensive, try disease.

INVESTMENT IN RESEARCH SAVES LIVES AND MONEY

Sickle Cell Disease

Sickle cell disease (SCD) is a group of inherited disorders that cause red blood cells to have abnormal hemoglobin, the molecule that transports oxygen. While red blood cells are supposed to be disc-shaped, the red blood cells of SCD patients "sickle" into crescent shapes, no longer moving easily through blood vessels. This can cause debilitating and deadly symptoms, including chronic pain, life-threatening bacterial infections, organ damage, and stroke. Individuals with sickle cell trait (SCT) carry one copy of the sickle cell disease gene, but do not usually experience symptoms.[1]

TODAY

It is estimated that

1 in 13

African Americans are born with SCT, and

1 in 365

with SCD.[1]

An estimated

100,000

Americans are living with SCD.[1]

If two individuals with SCT have a child, there is a **25%** chance the child will have SCD and a **50%** chance they will have SCT.[1]

75%

of adults with SCD do not receive hydroxyurea, the medication recommended by the National Heart, Lung, and Blood Institute to reduce the frequency of painful episodes.[2]

Research Delivers Solutions

Young children with SCD who receive a daily dose of **hydroxyurea** experience **less pain**, require **fewer blood transfusions** and are less likely to be **hospitalized** than children with SCD who do not receive hydroxyurea. The treatment was also associated with a 31% **reduction in hospitalization costs**, and a 21% net **decrease in annual direct medical costs** – a savings of approximately $3,000 per child.[7]

In clinical trials of **L-glutamine**, a **new treatment for SCD**, patients experienced **decreased hospitalization rates** and 14.5% **fewer instances of acute chest syndrome**.[6]

While **bone marrow transplants** offer a cure for SCD patients who can find a **compatible donor**, the **gene editing technique CRISPR** provides hope for the development of a more **universal cure**. CRISPR technology has been used to create a **gene therapy** treatment that **corrects the genetic mutation** that causes SCD patients to produce abnormal hemoglobin. Patients in this experimental trial now produce **normal hemoglobin**, and have **healthy red blood** cells.[8]

COST

$1.1 billion:

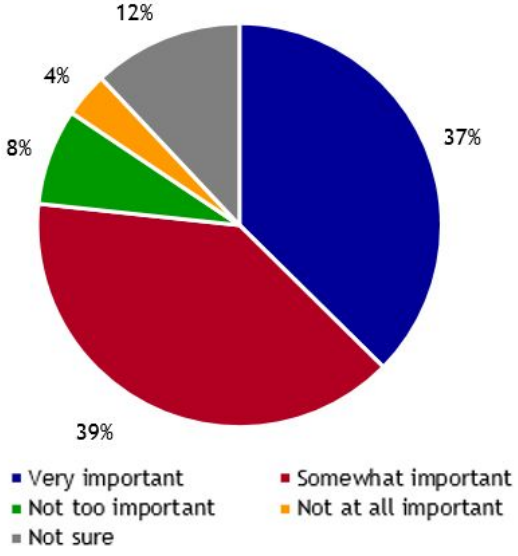
That's the total annual cost of direct medical care for individuals with SCD in the U.S.[4]

$1,049:

That's how much Medicaid spends per year on prescriptions and medications for each child with SCD.[5]

Majority Support Federal Incentives for Private Investment in New Treatments & Cures

How important is it for the federal government to incentivize greater private sector investment in new treatments and cures?



Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in January 2019

Sickle Cell Disease

Then. Now. Imagine.

THEN
In 1973, the average lifespan for an individual with SCD was 14 years.[9]

NOW
With improved screening techniques and medication, individuals with SCD live into their forties or fifties, and beyond.[10] Gene therapy and other avenues of discovery hold promise for even greater progress against this formidable health threat.

IMAGINE
If SCD was part of our past – not our future.

The average annual cost for SCD care is

more than

$10,000

for children and

$30,000

for adults.[3]

Spotlight on Advocacy

Adrienne Shapiro - Founder and Science Administrator, Axis Advocacy

From an early age, Adrienne Shapiro believed that "**science would solve sickle cell disease**." Adrienne's mother recognized the importance of testing for SC – even before newborn screening procedures had been implemented – and when her brother tested positive for SCD, the entire family learned how to treat and care for those living with the disease. Though Adrienne was told she did not carry SCT, the test she had received proved inaccurate and her daughter was diagnosed with SCD during a **newborn screening**.

Following her daughter's diagnosis, Adrienne began closely **monitoring progress in SC research**. She also began to work with other families by **hosting support group meetings**, and became a **strong advocate for research**. However, it was an invitation to the **World Stem Cell Summit** that truly introduced Adrienne to the power of science and advocates to finally cure SC.

Adrienne founded **Axis Advocacy** in 2016 to serve as a **clearinghouse for research** and help patients find and enroll in **clinical trials**. In addition to helping researchers **design better clinical trials for patients** and advocating for services that address the broad array of health concerns associated with SC, Axis takes a "**point of care**" approach to advocacy – going to meet patients where they are and working with medical teams to provide the best care.

A belief that **science holds the answers** and a commitment to **treating SC as a continuum** from childhood to adulthood – with challenges and needs changing along the way – inspires Adrienne in her efforts to **secure better care** for more people. "**In America, I want individuals living with SC to be treated according to evidence-based protocols so they can be alive and cure ready once we have new treatments**."

1. "Sickle Cell Disease." NHLBI, 2019
2. Stettler, et al. "Proportion of Adults with Pain Crises." 2015
3. "State of Sickle Cell Disease: 2016 Report" ASH, 2016
4. Kauf et al. "The Cost of Healthcare for Children." 2009
5. Mvundura et al. "Health Care Utilization and Expenditures." 2009
6. "FDA Approves New Treatment for Sickle Cell Disease." FDA, 2017
7. Wang et al. "Hydroxyurea Associated with Lower Costs." 2013
8. "SC Patient's Recovery after Gene Therapy." NHLBI, 2019.
9. McKerrell et al. "The Older Sickle Cell Patient." 2004.
10. "A Century of Progress: SCD." NHLBI, 2010.

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