The Cost:

- The total cost of direct medical care for individuals with SCD is $1.1 billion annually. 
- The average annual cost of SCD care is more than $10,000 for children and $30,000 for adults. By age 45, those with SCD reach an estimated total lifetime healthcare cost of nearly $1 million. 
- On average, Medicaid spends $1,049 per year on prescriptions and medications for each child with SCD. 

Perspective:

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.”

**Investment in research saves lives and money**

**Sickle Cell Disease**

Sickle cell disease (SCD) is a group of inherited disorders involving an abnormality known as hemoglobin S or sickle hemoglobin in the red blood cells. Sickle cell anemia and other forms of SCD can produce debilitating and deadly symptoms ranging from chronic pain to life-threatening bacterial infections to organ damage to stroke. **Individuals with sickle cell trait (SCT) carry the gene but do not experience symptoms.**

**Today:**
- An estimated 100,000 Americans are currently living with sickle cell disease (SCD). 1
- As many as 1 in 66 individuals (1.5%) born in the U.S. have sickle cell trait. 2 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 treatment recommended by the National Heart, Lung, and Blood Institute to reduce the frequency of painful episodes. 3
- Sickle cell gene mutations are especially common among African-Americans; it is estimated that 1 in 13 African-American babies are born with SCT and 1 in 365 with SCD. 1
- The only known cure for SCD is a bone marrow or stem cell transplant, both of which are limited by the availability of a genetically similar donor. 4

**How Research Saves Lives:**

- In 1973, the average lifespan for an individual with SCD was 14 years. 5 With improved screening and therapeutic approaches, the life expectancy of an individual with SCD is now between 40 and 60 years. 1
- Despite increased risk of infection, fewer than 50% of individuals with SCD receive the flu vaccine. In a National Institutes of Health (NIH) funded study, a pediatric SCD clinic at Boston University implemented several evidence-based approaches to increase vaccinations and raised the vaccination rate to 90% in just two flu seasons. 9
- Children with SCD are at a higher risk to develop, and subsequently die from, a bacterial infection known as invasive pneumococcal disease (IPD), compared to children without SCD. Due to the introduction of the pneumococcal conjugate vaccine, IPD rates for children with SCD dropped by 53%. 10
- In 2017, the FDA approved the drug Endari, the first new treatment for SCD in almost 20 years. Clinical trials found that individuals taking Endari experience a decrease in hospitalization rates and 14.5% fewer instances of acute chest syndrome. 11

**How Research Saves Money:**

- NIH-funded research at Johns Hopkins University found that 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, compared to children with SCD who did 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 for treating SCD – a savings of approximately $3,000 per treated child. 12, 13
- An NIH study found bone marrow transplants to be 87% effective in reversing the effects of SCD in patients. The average cost of a transplant is approximately the same as 10 sickle cell related hospital admissions – the average SCD patient experiences 6 admissions per year – making it a highly cost-efficient alternative. 14
- In 2016, researchers developed a low cost paper-based test that can detect SCT and SCD with over 90% accuracy in children aged one year or older. The test costs $0.77 to administer compared to standard blood tests that cost about $60. 15

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1 National Heart, Lung and Blood Institute, “Sickle Cell Disease.”
2 Centers for Disease Control and Prevention, “Incidence of Sickle Cell Trait.”
3 Stettler, N., et al., “Proportion of Adults with Sickle Cell.”
6 Wang, W.C., et al., “Hydroxyurea is Associated with Lower Costs.”
10 “Invasive Pneumococcal Disease among Children.”
14 Stettler, N., et al., “Proportion of Adults with Sickle Cell.”
15 “Invasive Pneumococcal Disease among Children.”
Hope for the Future:

Using the gene editing technique, CRISPR, NIH-funded researchers at Johns Hopkins University were able to collect red blood cells from individuals with SCD and correct the mutation in the cells. While this technique has not yet been tested in patients directly, the results provide hope for developing a “universal” cure for this life-threatening disease. 16

Treatment of vaso-occlusion episode (VOE), a common and painful symptom of SCD, has remained relatively unchanged over the past 80 years, focusing on pain management during the episode. An investigational drug, rivipansel, which is now moving to phase 3 clinical trials, showed encouraging results as a more effective means of addressing the pain.17

Preliminary laboratory studies conducted at Albert Einstein College of Medicine has uncovered a possible approach to prevent organ failure in SCD. The results suggest that certain antibiotics could reduce these common long-term complications. Additionally, the treatment was also shown to reduce VOE.18

SCD is the most common cause of strokes in children.19 Studies suggest that combinations of screening for blood flow in the brain, known as Transcranial Doppler (TCD) ultrasonography,20 blood transfusions and hydroxyurea reduce the likelihood of strokes in high-risk children.21

The Bottom Line:

SCD is a chronic, debilitating and life-threatening inherited condition with no standard treatment or universal cure.

As knowledge continues to grow around the possibilities of gene editing, stem cells and bone marrow transplants, researchers are closer than ever to developing a broadly effective cure for individuals with SCD. For this goal to become a reality, continued research investment is crucial.

Incidence of Sickle Cell Trait in 2010, by state
Incidence per 1,000 newborns screened at birth

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19 The Internet Stroke Center, “Stroke in Children with Sickle Cell Disease.”
21 NIH Research Portfolio Online Reporting Tools, “Sickle Cell Disease.”