



Sickle Cell Disease Fact Sheet

REQUEST: Cosponsor legislation to enhance federal government activities in sickle cell disease SCD research, training and services.

Sickle Cell Disease (SCD) and Sickle Cell Trait (SCT)

SCD is an inherited, lifelong disorder affecting nearly 100,000 Americans. Individuals with the disease produce abnormal hemoglobin which results in their red blood cells becoming rigid and sickle-shaped and causing them to get stuck in blood vessels and block blood and oxygen flow to the body. SCD complications include severe pain, stroke, acute chest syndrome (a condition that lowers the level of oxygen in the blood), organ damage, and in some cases premature death.

Sickle cell trait (SCT) is **not** a disease. Having SCT simply means that a person carries a single gene for sickle cell disease (SCD) and can pass this gene along to their children. People with SCT usually do not have any of the symptoms of SCD and live a normal life.

State of SCD Care and Research

- Although the molecular basis of SCD was established many decades ago, it has been challenging to translate this knowledge into the development of novel targeted therapies.
- New approaches in managing this disease have improved diagnosis and supportive care over the last few decades, but many patients still have severe complications to overcome.
- SCD patients encounter major issues accessing high quality care. Because SCD is a complex illness that affects multiple organ systems, few physicians specialize in SCD or can assume primary responsibility for a patient's care. Coordinated treatment continued across multiple settings is essential for patients to receive adequate care.
- With funding from the CDC Foundation, the Centers for Disease Control and Prevention (CDC) has established a population-based surveillance system to collect and analyze longitudinal data about people living in the U.S. with SCD. Due to limited funding, however, implementation of the program has occurred only in two states – California and Georgia (approximately 10% of the U.S. SCD population). Data is being collected from multiple sources (newborn screening programs and Medicaid) in order to create individual healthcare utilization profiles.

Steps Needed to Bridge the Gap in Care and Research

There is a critical need to improve outcomes for patients suffering with this disease. Expanded surveillance is necessary to improve understanding of the health outcomes and health care system utilization patterns of people with SCD, to increase evidence for public health programs, and to establish cost-effective practices to improve and extend the lives of people with SCD. Given the exciting and promising new SCD research announced at the December 2016 American Society of Hematology (ASH) Annual Meeting, now is the time to make the investment to ensure that patients have access to state-of-the-art clinical care.

Strengthening and expanding current efforts will help enable individuals living with this disease receive adequate care and treatment. Legislation has been introduced in the House by Representatives Danny Davis (D-IL) and Michael Burgess (R-TX) – H.R. 2410, the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2017 – that seeks to enhance federal government activities in sickle cell disease SCD research, training and services. Specifically, the legislation reauthorizes SCD prevention and treatment grants awarded by the Health Resources and Service Administration (HRSA), authorizes the CDC to award SCD surveillance grants to states, and authorizes research to expand the understanding of the cause and find a cure for SCD. Bipartisan companion legislation is expected to soon be introduced in the Senate.

The American Society of Hematology (ASH) represents more than 17,000 physicians, researcher, and medical trainees committed to the study and treatment of blood and blood-related diseases. ASH members include clinicians who specialize in treating children and adults with SCD and researchers who investigate the causes and potential treatments of SCD manifestations. ASH is committed to addressing the burden of SCD and recently released a *Call to Action on SCD* along with other stakeholders, founded the Sickle Cell Disease Coalition, and a public relations campaign. ASH's *State of SCD 2016 Report* and Report Card identified outlines the most pressing areas of need and provides a blueprint to advance these actions related to access to care, research and clinical trials, and global issues in sickle cell disease (SCD). For more information about the report, the report card and the new Sickle Cell Disease Coalition visit www.scdcoalition.org and for more information on SCD visit ASH's website (www.hematology.org/SCD).