The Sickle Cell Disease Association of America, Inc.

2015 Legislative Briefing Materials

Contact Organization
Sickle Cell Disease Association of America, Inc.
3700 Koppers Ave, Suite 570
Baltimore, Maryland 21227
P: 410.528.1555
F: 410.528.1495
www.sicklecelldisease.org

Points of Contact
Christopher Hollins, Chairman of the Board
chollins@sicklecelldisease.org

Sonja, L. Banks, President & COO
sbanks@sicklecelldisease.org

Dr. Kim Smith Whitley, Chief Medical Officer
kwhitley@sicklecelldisease.org

WHAT IS SICKLE CELL DISEASE?

Sickle Cell Disease (SCD) is an inherited blood disorder which constitutes a global public health problem. **At least 100,000 Americans live with the disease**. Many more Americans - as many as 2.5 million – including 1 in 12 African-Americans, are carriers for Sickle Cell (also called sickle cell trait or SCT). SCD affects African Americans, Hispanics and is common among those whose ancestors come from sub-Saharan Africa; regions in the Western Hemisphere; Saudi Arabia, India; and Mediterranean countries.

SCD causes the destruction of red blood cells due to the presence of the abnormal hemoglobin S, resulting in anemia and vaso-occlusion (blocking of blood vessels by sticky and inflexible red blood cells).

Complications include early childhood death from infection, stroke in young children and adults, infection of the lungs similar to pneumonia, pulmonary hypertension, chronic damage to organs such as the kidney resulting in chronic kidney failure, and frequent severe painful episodes. These unpredictable, intermittent, devastating pain events can begin as early as six months of age and can span a lifetime, impacting school attendance and the work force.

While the physical aspects of SCD are formidable, advances in the reduction of morbidity and mortality have provided the opportunity for a person with the condition to experience a prolonged and improved life.

However, there remains a significant lack of progress in the broader public health and social issues of educational, vocational, support for personal, family and community relations, practitioner and public education and mental health aspects of the lives of these individual. There is an emerging body of promising experimental and practice-based research that suggest improvements in the overall quality of life for persons with SCD and their families can be achieved. However, the relative lack of resources for such efforts remains problematic.

Federal resources directed to surveillance, research, newborn screening, follow-up, and ongoing treatment strategies such as analgesics and the medication hydroxyurea have been key to recent advances in fighting SCD.

SICKLE CELL DISEASE QUICK FACTS

- SCD affects an estimated **100,000** Americans
- **1,800 – 2,000** infants are born with SCD each year in the US, primarily African American and Hispanic
- SCD patients experience **18,000 – 20,000** or more blood transfusions per year
- Individuals living with SCD average **200,000** emergency department visits annually
- People with the most severe form of SCD may have a **2-3 decade** shorter life expectancy
- **20%-30%** of children with SCD experience stroke, and cognitive impairment is common
- **65%-70%** of those with SCD are classified as low income or economically disadvantaged
- Approximately **3 million** people in the U.S. and **300 million** worldwide are carriers
- Worldwide over **300,000** infants are born with SCD each year

BROAD REAUTHORIZATION PRIORITIES

In general, reauthorization efforts should focus on cohesive, multi-agency collaboration to promote continuity of care throughout the nation, while providing a basis for programmatic extension globally. To achieve this goal, SCDAA has identified the following broad reauthorization priorities.

- Swift reauthorization of the SCTA prior to the conclusion of the first session of the 114th Congress
- Continuation of existing authorization with modifications intended to improve patient care and quality of service
- Authorization of existing programs not already authorized (i.e., the SCD Community Outreach Demonstration Project and Hemoglobinopathies Data System)

PROPOSED MODIFICATIONS TO EXISTING TREATMENT ACT

- Continued authorization for community-based
- organization (CBO) **collaborative programs to improve medical and support services** delivered to affected individuals, and expanded eligibility beyond Federally Qualified Health Centers (FQHC)
- **Reconceptualization of SCD FQHC sites as Sickle Cell Treatment Centers (SCTC), to serve persons from age 6 and above,** using and applying best practices determined by CDC surveillance, NIH treatment research, and MCHB newborn screening research
- Specific direction in the Act to grantees to include the **development of transition services for adolescents to adult health care** as part of SCTC funded activity
- Authorization of HRSA to be the funding agency for SCTCs, and to determine the number of sites as needed based on population and geographic distribution of persons needing SCD treatment **(full funding for a minimum of 25 eligible SCTCs)**
- Continued authorization and continued support of a National Evaluation Center to collect, coordinate, monitor and distribute data, and to define best practice standards; development of treatment protocols and educational materials are anticipated
- Authorization of Centers for Disease Control (CDC) to be the funding agency for the **continuance and establishment of the Hemoglobinopathies Surveillance System program** and SCD public health promotion initiatives

WHY SUPPORT THE FIGHT AGAINST SCD!

- SCD is a major public health concern with an estimated healthcare impact of **$2 billion**
- SCD receives a significantly disproportionate amount of funding for research & treatment compared to other blood disorders or comparable diseases
- There is only **one (1) medication** that has been FDA approved to treat those with the disease
- There is an **overwhelming shortage** of specialists (primarily hematologists) that treat or specialize in SCD
- Many SCD adults **do not have** a medical home or primary care provider
- There is **no comprehensive model of care** within agencies to help reduce the major healthcare complexities that SCD patients encounter

NEW REAUTHORIZATION PROGRAMS AT A GLANCE

Sickle Cell Treatment Act Demonstration and Related Programs (Continuation & Expansion)
As enacted, the present Sickle Cell Treatment Act (Public Law 108-357) authorizes $10 million annually to support 40 demonstration projects and a National Coordinating Center. To date only nine (9) of these centers have been effectively established and are severely under-funded. We request within the new reauthorization the fully authorized amount of $10 million to fund twenty-five (25) treatment centers to be established for the purpose of supporting ongoing efforts to close gaps in medical service delivery, provider training and transitional care. Particularly as patients continue to cope with a struggling national economy these gaps currently impede access to care for many Americans affected by SCD.

Sickle Cell Disease Outreach and Newborn Screening Program (SCD-NBS) (Continuation & Expansion)
The goal of SCD-NBS is to enhance SCD materials, newborn screening and locally based outreach and counseling efforts for children with SCD and their families. The initiative's intent is to develop models that will enhance access to comprehensive care for such newborns and families, relying on partnerships between State Title V and newborn screening programs, local community-based SCD organizations, SCD treatment specialists, and community-based primary care providers.

Through past funding provided by Congress, two key components of this comprehensive effort have been established: (1) Six (6) demonstration sites across the country that provide SCD follow-up and other outreach services to support the comprehensive care of newborns diagnosed with SCD or sickle cell trait; and, (2) a National Coordinating Center that provides monitoring, coordination, support and information exchange in collaboration with these community organizations. $2.5 million is requested through the Maternal Child Health Bureau (MCHB). FY14 funding is intended to support the continuance and expansion of information dissemination and replication of best practices nationally.

Hemoglobinopathies Surveillance System (HSS) & SCD Public Health Initiatives (New)
The goal of this initiative is to establish a Hemoglobinopathies Surveillance System that will be used to describe the epidemiology and clinical characteristics of SCD, thalassemia, and other hemoglobin disorders in the U.S. The proposed HSS will assist national efforts to reduce mortality and disability due to hemoglobinopathies in the U.S. This data will also be used for research, information dissemination, policy decisions, and health care planning at the local, state, and national levels. Past funding for the HSS initiative was provided directly to Centers for Disease Control (CDC) through NIH as a two year pilot which ended in 2012.

SCDAA is committed to working with the CDC and seeks designated funding and authorization of the HSS program at the CDC, funded through the National Center for Preparedness, Detection, and Control of Infectious Diseases and requests $5 million in FY14 to support the continuance of the HSS.

SCD Research and Development Program (New)
SCDAA request $2.5 million to support programs to be established at the Health Resource and Service Administration (HRSA) for the purpose of sickle cell disease research evolving through patient care and outreach.

FUNDING REQUEST SUMMARY

Program	Requested Funding	Subcommittee	Agency
Sickle Cell Treatment Act Demonstration and Related Program	$10 Million	Labor-HHS-Education	Health Resources and Services Administration/ Bureau of Primary Care and Maternal Child Health
Sickle Cell Disease and Newborn Screening Program (SCD-NBS)	$2.5 Million	Labor-HHS-Education	Health Resources and Administration/Maternal Health Block Grant, Special Projects of Regional and National Significance (SPRANS)
Hemoglobinopathies Surveillance System (HSS) & SCD Public Health Initiatives	$5 Million	Labor-HHS-Education	Centers for Disease National Center for Preparedness, Detection and Control of Infectious Disease (Division of Healthcare Quality Promotion)
SCD Research and Development Programs	$2.5 Million	Labor-HHS-Education	Health Resources and Services Administration