Testimony of Sonja L. Banks

President, Sickle Cell Disease Association of America

"Examining Legislation to Improve Public Health"

Energy & Commerce Subcommittee on Health

September 8, 2016

Summary

Historical Background

I. Current State of Federal Law: Congress enacted and President Nixon signed into law the *Sickle Cell Anemia Control Act* in 1972. At the time, President Nixon pledged that his Administration would "reverse the record of neglect on the dreaded disease." Under that first law, Congress authorized $10 million to expand sickle cell programs in fiscal year 1972, with the goal of increasing funding in FY1973 and expanding SCD-related activities at the Veterans Administration.

Over 30 years later, Congress and President George W. Bush enacted the *Sickle Cell Treatment Act of 2003* (SCTA) as an amendment to the *American Jobs Creation Act of 2004* (P.L. 108-357). Original cosponsors Senator Charles Schumer (D-NY), Senator Richard Burr (R-NC) and Representative Danny Davis (D-IL) were instrumental in introducing the SCTA within the Jobs Creation Act.

II. Problem: Authorization for the SCTA expired on September 30, 2009. As a result, funding for SCTA programs, while not entirely gone, has diminished in the more than seven years that the reauthorization for the Act's programs has not occurred. The reach and scope of current activities suffer significantly from under-funding at the national level, with only a maximum of 9-10 treatments centers/sites were ever funded.

H.R. 1807

While work is still progressing, the HRSA grant programs need to be reauthorized to assure program stability and allow for a broader reach into areas of the country where people with sickle cell disease are not being adequately served. HR 1807 will also authorize a much needed surveillance program in SCD and other outreach and education efforts through the CDC.

Key Points:

- New name "The Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2013"
- Allows states to receive federal funding for patient counseling, educational initiatives and community outreach programs
- Sets the groundwork for the development of up to 25 sickle cell treatment centers located across the country and establishes a National Coordinating Center for Sickle Cell Disease.
- Supports the continuance of a National Coordinating and Evaluation Center and (6) community-based demonstration sites that provide SCD follow-up and other services to support comprehensive care for newborns diagnosed with SCD.
- Expands the development of transition services for adolescents to adult health care
- Seeks authorization of Centers for Disease Control (CDC) to continue and establish a Hemoglobinopathies Surveillance System program and SCD public health promotion initiatives.

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Chairman Pitts and Ranking Member Green, on behalf of the Sickle Cell Disease Association of America, Inc. (SCDAA) it 45 member organizations, over 35 affiliate partners and 100,000 plus patients and families we represent I want to thank you for holding this hearing and allowing me the opportunity to testify in support of H.R. 1807, the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act. I also would like to thank Dr. Burgess, the lead Republican cosponsor of this legislation, and Rep. Butterfield, for their leadership on this important legislation. We would also be remiss if we did not recognize Rep. Danny Davis for not only leading this important legislation but remaining one of the sickle cell community's key champions.

I am here today representing the nation's only organization working full time on a national level to resolve issues surrounding sickle cell disease and sickle cell trait. Since 1971, the SCDAA has been on the forefront of improving the quality of health, life and services for individuals, families and communities impacted by sickle cell disease. Additionally, SCDAA has been and remains

instrumental in promoting policies and research facilitating the continued search for a “universal” cure for sickle cell disease.

Sickle cell disease is an inherited blood disorder affecting approximately 100,000 Americans. The disease causes the destruction and deformation of red blood cells due to the presence of abnormal hemoglobin, which results in anemia and vaso-occlusion, which essentially means the blocking of blood vessels due to sticky, inflexible, sickled - shaped red blood cells. One in every 400 African American newborns has sickle cell disease, as does one in every 1,200 newborns of Hispanic descent.

The consequences and complications of this disease are extreme. Common complications include early childhood death from infection, stroke in young children and adults, lung problems similar to pneumonia, chronic damage to organs including the kidney, leading to kidney failure, and to the lungs causing pulmonary hypertension, and severe painful episodes. In fact pain episodes are a hallmark of sickle cell disease. They are unpredictable in many ways, both the timing of when they occur, how severe they will be and how long they will last. For those with the disease, these devastating pain episodes can start as early as six months of age and can span a lifetime, impacting school attendance and participation in the workforce. In fact, these pain crises contribute significantly to the 200,000 emergency room visits collectively made by sufferers of sickle cell disease each

year in our country. A typical crisis will result in a hospital stay of seven to ten days. Due to the lack of effective treatment options to treat the disease, patients are given medication to ensure hydration and opioids to manage the pain. Although we have known for over five decades the gene responsible for sickle cell disease, we have no medications to alter the course of a pain episode. Sadly, people with the most severe form of the disease may have a two to three decade shorter life expectancy.

In 2004, the Congress recognized the need to do more to improve the treatment and prevention of sickle cell disease. Specifically, as part of the American Jobs Creation Act, a Sickle Cell Disease Prevention and Treatment Demonstration Program was enacted. The demonstration program required the Health Resources and Services Administration to make grants to up to 40 eligible entities for the purposes of developing and establishing systemic mechanisms to improve the prevention and treatment of sickle cell disease. Specifically, grantees were charged with improving the coordination of service delivery for patients; providing genetic counseling; training health professionals; working to ensure greater newborn screening; and working with state health departments to ensure access to care, among other things. To ensure this was done most effectively and comprehensively, the grants were to be awarded to heighten geographic diversity and grantee collaboration with sickle cell disease treatment centers. Additionally,

the legislation called for the creation of a National Coordinating Center for the demonstration program, and this Center is administered by the National Institute for Children's Health Quality.

While the enactment of the Sickle Cell Treatment and Prevention Demonstration Program was a major step forward for the individuals and families suffering with this disease, since that time the program has not been fully-funded and opportunities for advancement have been lost. As stated, while the law envisioned the creation of 40 eligible entities to develop and establish systemic mechanisms to improve the prevention and treatment of sickle cell disease, to date only nine grants have been given to eligible entities.

H.R. 1807 reauthorizes and improves upon the HRSA demonstration program. The treatment and prevention component reauthorization, contained within section 4 of the bill, sets a more realistic number of eligible entities which can be funded. The original law specified 40 eligible entities, H.R. 1807 sets that number at 25 eligible entities.

Importantly, a major advancement made in H.R. 1807 would place a duty on these grantees to "expand, coordinate, and implement transition services for adolescents with sickle cell disease making the transition to adult-focused health care." Today, so many young people fall through the cracks as they transition

from childhood to adolescence and young adulthood. This very important change would make it a requirement for grantees to adopt strategies to ensure that these individuals transition appropriately, minimizing the disruption of care and resulting in better health outcomes.

Additionally, H.R. 1807 establishes a new surveillance grant program for states, wherein grants would be authorized to up to 20 states representing a majority of the sickle cell disease patient population. The current surveillance conducted by the CDC is limited to the state of California and the data collected is general in nature. The data which would be accumulated under this grant program authorized by HR 1807 would cover associated health outcomes, complications and treatments, and would result in public health initiatives and strategies which would improve current estimates about the incidence and prevalence of the disease, would identify health disparities, would assess the utilization therapies and strategies to prevent complications from the disease, and would evaluate the impact of genetic, environmental, behavioral and other risk factors that may impact health outcomes.

SCDAA has been working in partnership with the American Society of Hematology, the world's largest society of professional Hematologists, in support of the reauthorization of the sickle cell disease programs in HR 1807 and on other legislative and regulatory initiatives to better meet the needs of this underserved

community. Earlier this week ASH released a report supported by more than 20 organizations evaluating and making recommendations related to multiple aspects of sickle cell disease, including access to care in the US, training and professional education, research and clinical trials, and global health. Together, private sector organizations along with federal and state government agencies working in a coalition will make a real difference in improving the lives of people with sickle cell disease.

Finally, SCDAA wants to recognize the important advancements being made to not only treat the symptoms of those suffering from sickle cell, but to treat the disease itself. It's been decades since the FDA has approved new treatments for the benefit of patients with sickle cell disease. During the debate on the 21st Century Cures Act, Rep. Rush noted the historic underrepresentation of minority communities in clinical trials, and the fact that the nation needs to do a better job of supporting institutions that educate medical professionals with an interest in working in minority communities. By doing this, we could make advancements in the development of therapies for diseases like sickle cell which primarily impact minority populations.

Nearly 80% of hospitalizations for sickle cell disease occur among those covered by public payers Medicaid and Medicare." The good news is, as it relates specifically to sickle cell disease, we are on the precipice of exciting new therapies which hold the promise of lessening patient suffering and reliance on expensive acute care. These therapies could be approved by FDA as soon as 2018. More importantly these therapies may improve the quality of life of those living with sickle cell disease. The SCDAA would like to work with this committee and the Congress to ensure that when these therapies, which will alleviate so much pain and suffering for patients with sickle cell disease, are approved, there are incentives for hospitals to provide these drugs to patients. We cannot have a situation develop where a breakthrough sickle cell disease drug is approved for the benefit of patients in crisis, but there are disincentives for hospitals to use these new therapies. There are government programs, such as the New Technology Add-On Payment program in Medicare, which attempts to ensure that new therapies are available for needy, hospitalized patients, but only a handful of drug therapies have ever been approved for this program. And some have found that the program doesn't do enough to incentivize hospitals to use the new therapies. And no such program exists under the Medicaid program, which provides coverage for so many sickle cell disease patients. The presence of new, breakthrough therapies

is a tremendous advance, but we must ensure that disease sufferers have access to these therapies when they are in need.

Thank you very much for allowing me to testify before you today.