



**American Society of Hematology
Statement to the
Committee on Energy and Commerce
Subcommittee on Health**

Hearing on Examining Legislation to Improve Public Health

September 8, 2016

Chairman Pitts, Ranking Member Green, and members of the Energy and Commerce Subcommittee on Health, the American Society of Hematology (ASH) is extremely grateful for the opportunity to provide written testimony for the record on H.R. 1807, the *Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2015*, which was introduced last year by Representatives Danny Davis and Michael Burgess. We applaud their efforts along with those of members of the Congressional Sickle Cell Disease Caucus.

ASH represents more than 16,000 physicians, researchers, and medical trainees committed to the study and treatment of blood and blood-related diseases such as leukemia, lymphoma, and myeloma; non-malignant conditions, including anemia and hemophilia; and congenital disorders, such as thalassemia. ASH members also include clinicians who specialize in treating children and adults with sickle cell disease (SCD) and researchers who investigate the causes and potential treatments of sickle cell disease manifestations.

SCD is an inherited, lifelong blood disorder that causes individuals to produce abnormal hemoglobin, causing their red blood cells to become rigid and sickle-shaped. These sickled cells have a shortened lifespan, resulting in a constant shortage of red blood cells. When these sickled cells travel through the blood, they often get stuck in the smaller blood vessels, blocking other oxygen-rich red blood cells from freely flowing throughout the body. This leads to complications ranging in severity, including severe pain, acute chest syndrome (a condition that lowers the level of oxygen in the blood), stroke, organ damage, and even premature death. In the United States, approximately 100,000 individuals have SCD. The Centers for Disease Control and Prevention (CDC) estimates that SCD affects 1 out of every 365 black or African-American births, and 1 out of every 16,300 Hispanic-American births.

Although the molecular basis of SCD was established five decades ago, it has been challenging to translate this knowledge into the development of novel targeted therapies. Many important discoveries have been made, and some treatments developed. These discoveries have identified innumerable questions and opportunities to better understand and treat this complex disease. Yet, many basic scientific processes are still not fully understood, too few treatments have been developed, and care that could improve the duration and quality of life for individuals with SCD is inconsistent in the United States and wholly absent in large parts of the world.

Currently, Hydroxyurea, the only FDA-approved drug for adults with SCD (often used off-label in children), improves the course of SCD and might lead to significant health care cost reductions. In a two year pediatric study, overall health care costs for children on hydroxyurea were \$1.8 million, compared with \$2.5 million for those who did not receive this treatment.

Unfortunately, hydroxyurea is not regularly prescribed and adherence to the therapy is poor. In addition to hydroxyurea, blood transfusions can help to manage SCD, but they can lead to abnormally high levels of iron in the blood, which can cause long-term organ damage and reactions due to a mismatch between the donors and recipients. There has also been early success in curing SCD through bone marrow transplant, but this process is costly, dangerous, and is not widely available.

In an effort to identify unmet medical needs for people with SCD, ASH, along with other groups, earlier this week issued the [State of Sickle Cell Disease: 2016 Report](#), evaluating the disease in four priority areas — access to care, training and professional education, research and clinical trials, and global health. The report shows that significant improvements are needed across all areas and that, though patients are living longer, the system of care needs to change to ensure a better quality of life. To address these challenges, ASH launched the [Sickle Cell Disease Coalition](#) along with more than 20 other organizations who are issuing a call to action that will amplify the voice of the SCD community, promote SCD awareness, and transform SCD care both in the United States and around the globe.

ASH thanks the Subcommittee for focusing needed attention on SCD and supports *The Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2015* (H.R. 1807). However, the Society believes there is the need for additional legislation to enhance current federal activities in SCD research, training and services.

Strengthening and expanding current efforts will help ensure that individuals living with this disease receive adequate care and treatment. In fact, a multi-pronged approach by key Federal agencies in collaboration with private sector sickle cell stakeholder groups could more efficiently, effectively and economically improve the comorbidities and health disparities faced by individuals with SCD and their families. Specifically, ASH believes the following priorities are important components of any legislative package addressing SCD:

- Authorization of the Department of Health and Human Services' Interagency Working Group on SCD to coordinate efforts among federal agencies.
- Enhancement of the Centers for Disease Control and Prevention's SCD outreach and education programs on SCD and SCD trait for patients and providers.
- Improvement of access to high quality care via demonstration projects at CMS and development of best practices.
- Provision of incentives for drug development for SCD within the Food and Drug Administration (H.R. 1537, Advancing Hope Act of 2015).

The status quo is unacceptable, and we must improve the circumstances under which care for individuals with SCD is provided. Now is the perfect time – today, we have opportunities to improve overall treatment, care, and quality of life for millions of people, especially young children. We are calling for action in sickle cell.

Thank you again for the opportunity to submit testimony. Please contact Stephanie Kaplan, ASH Senior Manager of Government Relations and Public Health, at 202-776-0544 or skaplan@hematology.org, or Tracy Roades, ASH Legislative Advocacy Manager, at 202-776-0544 or troades@hematology.org, if you have any questions or need further information.