Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2017 (HR 2410, 115th Congress)

Amends the Public Health Service Act to authorize sickle cell surveillance, prevention, and treatment initiatives and to establish conditions for collaboration with community-based organizations on such initiatives.

Updated last **August 3, 2017**
for the 05/11/2017 version of HR 2410.

WHAT IT DOES

The Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2017 (HR 2410) is designed to reauthorize expired federal sickle cell disease programs and establish new federal sickle cell disease research, surveillance, prevention, and treatment programs.

HR 2410 amends section 399V of title III of the Public Health Service Act (42 U.S.C. 280g-11 *et seq.*) to authorize the Department of Health and Human Services to undertake the following research and surveillance efforts:

- Establish research efforts to explore causes and cures for the disease.
- Award grants to entities in up to 20 states that demonstrate collaboration with other communal organizations and healthcare providers to:
  - Collect data on the prevalence and demographics of sickle cell disease;
  - Organize public health initiatives that increase access to health care, increase knowledge of recommended prevention and screening strategies, increase knowledge of recommended treatment, promote consistent guidelines for medical care, train healthcare providers and organizations, train state health departments, and educate communities at large; and
  - Research potential prevention and treatment strategies, particularly focusing on factors that contribute to the cause of the disease (including genetic factors), determine disease disparities, and affect disease outcomes.

HR 2410 also amends the American Jobs Creation Act of 2004 to reauthorize funding for the treatment program outlined in Section 712(c) (42 U.S.C. 300b–1 note), also known as the Sickle Cell Disease Treatment Demonstration Program, which attempts to coordinate sickle cell disease treatment efforts. The amendment reauthorizes the program and makes the following alterations to the program's jurisdiction:

- Award grants to up to 25 entities per year;
- Expand resources for adolescents with sickle cell disease; and
- Award $4,455,000 for program funding per year, up through FY2022.

RELEVANT SCIENCE

Sickle cell disease (SCD) describes a group of genetic blood disorders. According to the Centers for Disease Control and Prevention (CDC) around 100,000 Americans have SCD. The CDC further explains that those with SCD are primarily of African descent but that SCD also affects those from South America, Central America, the Caribbean, and the Mediterranean.

The National Institute of Health (NIH) explains that SCD affects red blood cells by affecting hemoglobin, the protein in red blood cells that transports oxygen through the body. The hemoglobin protein is comprised of four protein subunits, two of which are called beta-globin and are produced by the HBB gene. Each person has two HBB genes, one inherited from each parent. Some mutations in the HBB gene produce an abnormal beta-globin that functions improperly. For people who develop SCD, both HBB genes are thus

mutated and at least one produces an abnormal beta-globin called hemoglobin S (HbS). The second HBB mutation also produces an abnormal beta-globin, either HbS or another variant like hemoglobin C (HbC), D (HbD), E (HbE), O (HbO), or β (Hbβ). This variable second mutation defines the distinction between various forms of SCD. The most severe form of SCD, sickle cell anemia (HbSS), occurs when individuals inherit copies of the HbS gene from both parents, and thus are only able to produce hemoglobin S.

According to the National Heart, Lung, and Blood Institute of the NIH, inheriting mutated hemoglobin genes alters the functional capacity of red blood cells. Red blood cells with normal hemoglobin are round, giving them a flexibility that allows them to circulate through blood vessels of all sizes and transport oxygen throughout the body. Abnormal hemoglobin can stick together to form long rods that cause red blood cell to be sickle shaped and inflexible. This inflexibility has two primary consequences:

- Less blood flow: Blood cells are more easily caught on the sides of blood vessels, either slowing the flow of blood or stopping it entirely. The lack of red blood cells in parts of the body can cause:
  - Acute and chronic pain;
  - Swelling;
  - Delayed growth; and
  - Organ complications, particularly in the liver, kidney, heart, and eyes.
- Anemia: Blood cells have a shorter life span because they burst easily. The lifespan of sickle cells is 10 to 20 days instead of the normal 90 to 120 days, making it difficult for the body to produce enough blood cells to keep up with blood cell turnover rate.
- Other complications include increased infections, liver failure, brain development problems, kidney problems, heart disease, priapism, gallstones, ulcers, delayed puberty, and pregnancy problems.

Mutations in HBB are the primary cause of SCD. However, as evidenced by the diversity in symptoms between people with the same mutations, other genetic factors are likely to play a less direct role as modifiers of the disease. Many of these genetic factors are unknown or modify SCD through unknown pathways. Identifying and understanding the heritable genetic factors that affect expression of SCD could lead to improvements in treatments and general health care.

RELEVANT EXPERTS

Soheir Saeed Adam, MBBCh, is an Assistant Professor of Medicine at the Duke University School of Medicine. Her research focuses on physical markers of sickle cell disease.

Relevant Publications:

- Amin, C, Adam, S, Mooberry, MJ, Kutlar, A, Kutlar, F, Esserman, D, Brittain, JE, Ataga, KI, Chang, J-Y, Wolberg, AS, and Key, NS. 2015. "Coagulation Activation in Sickle Cell Trait: An Exploratory Study." *British Journal of Haematology* 171(4):638-646. doi: 10.1111/bjh.13641
- Adam, SS, and Hoppe, C. 2013. "Potential Role for Statins in Sickle Cell Disease." *Pediatric Blood Cancer* 60(4): 550-557. doi: 10.1002/pbc.24443
- Adam, SS, Flahiff, C, Abrams, MR, Telen, MJ, and De Castro, LM. 2009. "The Complex Relationship Between Sickle Cell Disease and Depression." *Blood Journal* 114(22): 2585.
- Jonassaint, JC, Jonassaint, CR, Flahiff, CM, Ball, A, Adam, SS, Telen, MJ, and De Castro, LM. 2008. "Does Living Closer to a Medical Care Center Matter in Sickle Cell Disease?" Blood Journal 112(11): 323-324.
- Adam, S, Jonassaint, J, Kruger, H, Kail, M, Orringer, EP, Eckman, JR, Ashley-Koch, A, Telen, MJ, and De Castro, LM. 2008. "Surgical and Obstetric Outcomes in Adults with Sickle Cell Disease." *American Journal of Medicine* 121(10):916-921. doi: 10.1016/j.amjmed.2008.04.040

Regina Denise Crawford, MD, is an Assistant Professor of Medicine at the Duke University School of Medicine. Her research involves studying the cognitive functions of those with sickle cell disease.

Relevant Publications:

- Crawford, RD, and Jonassaint, CR. 2016. "Adults with Sickle Cell Disease May Perform Cognitive Tests as Well as Controls When Processing Speed is Taken into Account: A Preliminary Case-Control Study." *Journal of Advanced Nursing* 72(6): 1409-1416. doi: 10.1111/jan.12755
- Feliu, MH, Crawford, RD, Edwards, L, Wellington, C, Wood, M, Whitfield, KE, and Edwards, CL. 2011. "Neurocognitive Testing and Functioning in Adults Sickle Cell Disease." *Hemoglobin* 35(5-6): 476-484. doi: 10.3109/03630269.2011.626098

Marilyn Jo Telen, MD, is a Professor of Medicine and Associate Professor of Pathology at the Duke University School of Medicine. She is also Director of the Duke Comprehensive Sickle Cell Center. Her research involves studying the adhesion receptors of cell membrane proteins to understand the interaction of these proteins with red blood cells and other proteins in sickle cell disease.

> "Despite a vastly improved understanding of how sickle hemoglobin causes disease, slow progress has been made in development of new effective therapies, including medicines, bone marrow transplantation and gene therapy, the latter two being the only ones offering a potential cure for the disease. However, in the last few years, significant progress has been made on all three fronts, so that now is the time to truly push forward to perfect and complete these therapies. Two drugs have shown promise in early clinical trials, bone marrow transplantation has apparently cured small number of patients, and at least one patient has responded to gene therapy. However, given the low numbers affected by SCD in the US, pharmaceutical and biotech companies have a limited financial incentive to develop such therapies."

Relevant Publications:

- Telen, MJ. 2016. "Beyond Hydroxyurea: New and Old Drugs in the Pipeline for Sickle Cell Disease." *Blood Journal* 127(7): 810-819. doi: 10.1182/blood-2015-09-618553
- Telen, MJ. 2015. "Biomarkers and Recent Advances in the Management and Therapy of Sickle Cell Disease." *F1000 Research* 4.
- Elmariah, H, Garrett, ME, Soldano, KL, Ataga, KI, Eckman, JR, Telen, MJ, and Ashley-Koch, AE. 2014. "Genes Associated with Survival in Adult Sickle Cell Disease." Blood Journal 124(21): 2719.
- Elmariah, H, Garrett, ME, De Castro, LM, Jonassaint, JC, Ataga, KI, Eckman, JR, Ashley-Koch, AE, and Telen, MJ. "Factors Associated with Survival in a Contemporary Adult Sickle Cell Disease Cohort." American Journal of Hematology 89(5): 530-535. doi: 10.1002/ajh.23683

BACKGROUND

While symptoms of sickle cell disease were observed as early as the seventeenth century, the first description and published case of sickle cell disease in the United States was not made until 1910. According to the American Society of Hematology (ASH), despite this discovery and the discovery of other cases of sickle cell disease in the following decades, research for the condition was widely neglected due to its high prevalence in individuals of African descent. The ASH furthers that it was not until the 1970s, thanks largely to the Civil Rights Movement, that there was a demand to raise national awareness around issues surrounding sickle cell disease and treat the disease. During this period, research on sickle cell disease increased and health organizations designed to address sickle cell disease were formed, such as the Sickle Cell Disease Association of America.

The National Sickle Cell Anemia Control Act, the first major federal law addressing sickle cell disease, was signed in 1972. This law increased federal spending to expand sickle cell disease programs by $10 million dollars, a tenfold increase of the existing spending budget for sickle cell disease programs, and expanded the sickle cell program of the Veterans Administration. Three decades later, the Sickle Cell Treatment Act of 2003 (SCTA) was signed into law. This law also increased grant programs for research and treatment of sickle cell disease, including the Sickle Cell Disease Treatment Demonstration Program. However, authorization for the SCTA expired in 2009 and there were no immediate laws enacted to reauthorize funding for the programs established under the SCTA.

The Sickle Cell Disease, Research, Surveillance, Prevention, and Treatment Acts of 2014, 2015, and 2017 are the first bills post-SCTA expiration that have attempted to reauthorize funding for SCTA established programs.

In addition to research and treatment oriented legislation, Congress has introduced resolutions designed to increase awareness about sickle cell disease. For example, a simple resolution seeking to create a "Sickle Cell Disease Awareness Month" was introduced in both chambers (H Res 903/S Res 592) in the 114th Congress. These resolutions would establish September as a month of awareness for sickle cell disease to promote public sickle cell disease knowledge Another resolution titled "Calling for Sickle Cell Research" (H Res 296) was introduced in the House in the 114th Congress. This resolution would encourage medical professionals to increase education and awareness on sickle cell disease and the Department of Health and Human Services to create public awareness campaigns about sickle cell disease.

ENDORSEMENTS & OPPOSITION

Endorsements:

- Representative Michael C. Burgess, MD explains how HR 2410 achieves the foundational necessity of increasing public awareness in a public statement: "Having cared for patients with sickle cell disease as a physician at Parkland Hospital, I've seen firsthand the devastating effect this disease can have on people. This bill provides an important step forward in ensuring that we have the resources to better understand this disease and to maintain access to services for those affected by sickle cell disease."
- Sonja Banks, President of the Sickle Cell Disease Association of America, a national organization working to promote research and education on sickle disease, expressed support for a previous iteration of the bill (HR 1807, 114th Congress) in a testimony in front of the Energy and Commerce Subcommittee on Health. Her testimony brought three main points of support to attention:
  - "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…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."
  - "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"

Opposition:

At present, there has not been any publicly reported opposition to this bill.

STATUS

HR 2410 was introduced in the House on May 11, 2017 and referred to the House Committee on Energy and Commerce on June 7, 2017. After referral to and consideration by the Committee's Subcommittee on Health, it was ordered to be reported by Voice Vote by the Committee on the same day.

RELATED POLICIES

There was no related legislation in the concurrent Congressional Session.

POLICY HISTORY

HR 2410 is the third version of the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act. The bill was previously introduced as the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2014 (HR 5124, 113th Congress). A nearly identical bill was then introduced as the Sickle Cell Disease Research, Surveillance, Prevention, and Treatment Act of 2015 (HR 1807, 114th Congress). Representative Danny Davis (D-IL-7) introduced all three versions of the bill in the House.

SPONSORS

Sponsor: Representative Danny Davis (D-IL-7)

Cosponsors: Representative Michael Burgess (R-TX-26)

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