COMMENTARY

blood advances

TO THE EDITOR:

Understanding sickle cell disease: impact of surveillance and gaps in knowledge

Mandip Kaur,[1] Mary Brown,[2,*] Ted W. Love,[3,*] Alexis Thompson,[4,*] Marsha Treadwell,[5,*] and Kim Smith-Whitley[6,*]

[1]CDC Foundation, Atlanta, GA; [2]Sickle Cell Disease Foundation of California, Ontario, CA; [3]Global Blood Therapeutics, San Francisco, CA; [4]Ann and Robert H. Lurie Children's Hospital of Chicago, Chicago, IL; [5]UCSF Benioff Children's Hospital of Oakland, Oakland, CA; and [6]Children's Hospital of Philadelphia, Philadelphia, PA

On 18 December 2018, the Sickle Cell Disease and Other Heritable Blood Disorders Research, Surveillance, Prevention, and Treatment Act of 2018 was signed by President Trump and became Public Health Law No. 115-327.[1] This new law creates Section 1106 of the Public Health Service Act, which allows for grants "to improve data on the incidence and prevalence of heritable blood disorders, including sickle cell disease (SCD), and the geographic distribution of such diseases and conditions."[2] In essence, it formalizes and codifies an expansion of the Centers for Disease Control and Prevention's (CDC's) Sickle Cell Data Collection (SCDC) program.[3] The SCDC program is a population-based, longitudinal surveillance system that incorporates information from multiple data sources, such as newborn screening; state Medicaid, hospital discharge, and emergency department claims; and vital records, to identify individuals living with SCD and to better understand their health outcomes and patterns of healthcare use. National surveillance for SCD would provide the data needed to help healthcare providers understand how patients interact with the healthcare system and how these factors affect health in the short and long terms; how researchers and public health professionals know where to target activities and programs that will result in improvement in health care, including better access to SCD clinical centers; and how policymakers and administrators assign resources and assess programs to improve healthcare services and overall patient health.

Some of the most fundamental gaps in knowledge that the SCDC program aims to fill are a better understanding of how many people have SCD in the United States, where they live, and where they receive medical care. The infrastructure and design of the SCDC program ensure that almost all individuals living with SCD can be included in the system, regardless of where or when they were born, how sick they are, what kind of insurance they have, or where they receive care. The entire spectrum of the SCD population is captured because the SCDC program contains population-based data, rather than data from select health facilities or single data sources, that spans multiple years (currently 2004-2016). Comprehensive data are especially important in the case of SCD, as many individuals with the condition do not have access to healthcare providers who specialize in SCD and often rely on nonhematologists, or emergency department providers, for the majority of their care.[4]

Although improvements in healthcare and treatment during the last several decades have enabled people with SCD to live into adulthood, the most severe form of SCD can shorten the lives of people with SCD by 20 to 30 years.[5] These poor health outcomes are likely influenced by limited or no access to quality, comprehensive care, as there is a dearth of health professionals and facilities that care for the SCD community, especially adults[6]; limited access to treatments, including hydroxyurea, which is often underprescribed[7]; and mistaken beliefs about people with SCD, including doubts about patients' reports of pain.[8]

Not only is there a lack of awareness and comfort in caring for individuals with SCD among primary care providers but also a limited number of hematologists who specialize in SCD care,[9,10] and very few comprehensive SCD clinical centers (centers that provide multidisciplinary SCD services including primary care and mental health services) exist.[11] A number of efforts are currently being funded by agencies throughout the US Department of Health and Human Services and professional organizations to address these challenges, such as the Health Resources and Services Administration's Sickle Cell Disease Treatment Demonstration Program and Newborn Screening Program,[12] The National Institutes of Health's Sickle Cell Disease Implementation Consortium,[13] and the American Society of Hematology's Sickle Cell Disease Research Collaborative.[14] However, to increase access to quality care, these programs must first find patients who are not affiliated with existing SCD centers and

medical experts. By providing a better understanding of where these patients are and where they receive their care, the SCDC program can play a role in helping patients, communities, healthcare providers, policy makers, researchers, and federal, state, and local health agencies improve access to the healthcare system, understand more about ongoing clinical trials, and benefit from new treatments as they become available to the patient community.

The SCDC program has been operating in 2 states, California and Georgia, since 2015, and the benefits to the SCD community are numerous. In California, as a result of Assembly Bill 1105,[15] there are now appropriations in the state's 2020 budget to provide financial resources to create comprehensive networks of care across the state to provide better access for adults with SCD. SCDC data were used to help the bill's authors and advocates identify the state's areas of highest need for these SCD specialty care centers, including parts of the state that were previously unknown to have a large number of individuals with SCD. The community-based organization, Sickle Cell Disease Foundation of California,[16] is using SCDC data to identify and engage with primary care providers who are already taking care of individuals with SCD to both connect them with new patients and provide helpful resources for appropriate management of challenges that may arise as a result of the complexity of SCD. In Georgia, SCDC data have been used to identify locations with large numbers of individuals with SCD but no healthcare providers with expertise in SCD. These locations are targeted for new satellite clinics staffed by faculty from established comprehensive SCD clinical centers both in person and through telemedicine.[17]

SCDC data from California and Georgia are also adding to the medical and research communities' understanding of the clinical course of SCD. New findings on the incidence of SCD,[17] mortality rates and causes,[5] palliative care,[18] emergency department and hospital use,[19] insurance coverage,[20,21] and women's health have been presented,[22,23] and numerous analyses are currently underway. These results are incredibly useful, as they tell the complete story of all individuals with SCD in these 2 states, regardless of age, disease severity, insurance status, or location of health care.

Although the data from 2 states provides key information to understand health care and outcomes of people with SCD in each state, drawing conclusions about national outcomes requires significantly more information about patients across the country as healthcare systems and patient populations vary by state. The challenges that patients face in 1 state may not necessarily be the same in other states, and as a consequence, recommendations for interventions to improve health outcomes may also be different. As a result, because of the lack of Congressional funding for the CDC's sickle cell activities, the SCDC program is funded, almost exclusively, by public-private partnerships through the CDC Foundation.[24] Congressional appropriations in the FY20 Federal Budget for CDC to conduct the activities outlined in Public Health Law No. 115-327 would allow for the expansion of SCDC activities to additional states. Resources are needed to establish a national surveillance system to learn the exact number of people with SCD in the United States, to identify health disparities endured by the SCD population, and to provide the evidence needed to improve health outcomes for this condition. Without resources to sustain current activities and expand the SCDC program to additional states, our understanding of how new policies and new treatments affect access to care, health complications, health outcomes, and life expectancy will be eliminated, perpetuating health disparities among those living with SCD. But it is not just the SCD community that would be withheld from benefits: the state and federal governments, through Medicaid and Medicare, will continue to pay for the large majority of the SCD population's costly health care.[25]

Improving health outcomes for the SCD population will require a collaborative effort among patients, communities, healthcare providers, policy makers, researchers, and federal, state, and local health agencies. Although the scientific community is poised to dramatically change health outcomes for individuals with SCD, adequate funding for the SCDC program will be critical to reaching these individuals and their providers to implement these changes.

*M.B., T.W.L., A.T., M.T., and K.S.-W. contributed equally to this work.

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ORCID profiles: M.K., 0000-0003-3312-6075; A.T., 0000-0003-4961-8103; M.T., 0000-0003-0521-1846.

Correspondence: Mandip Kaur, CDC Foundation, Bank of America Plaza, 600 Peachtree St NE, Suite 1000, Atlanta, GA 30308; e-mail: wvx6@cdc.gov.

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